

Use these links to rapidly review the document

[TABLE OF CONTENTS](#)

[INDEX TO CONSOLIDATED FINANCIAL STATEMENTS](#)

[Table of Contents](#)

This draft registration statement has not been publicly filed with the Securities and Exchange Commission and all information herein remains strictly confidential.

As confidentially submitted to the Securities and Exchange Commission on February 13, 2018.

Registration No. 333-

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

**Form S-1**  
REGISTRATION STATEMENT  
UNDER  
THE SECURITIES ACT OF 1933

**Y-mAbs Therapeutics, Inc.**

(Exact name of registrant as specified in its charter)

<b>State of Delaware</b> (State or other jurisdiction of incorporation or organization)	<b>2834</b> (Primary Standard Industrial Classification Code Number)	<b>47-4619612</b> (I.R.S. Employer Identification Number)
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**Y-mAbs Therapeutics, Inc.**

**750 Third Avenue**

**9<sup>th</sup> Floor**

**New York, NY 10017**

**Tel. (212) 847-9841**

(Address, including zip code, and telephone number,  
including area code, of registrant's principal executive offices)

**Thomas Gad**

**Founder, Chairman, President and Head of Business Development**

**Y-mAbs Therapeutics, Inc.**

**750 Third Avenue**

**9<sup>th</sup> Floor**

**New York, NY 10017**

**Tel. (212) 847-9841**

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**Approximate date of commencement of proposed sale to the public:**  
**As soon as practicable after the effective date of this registration statement.**

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act, check the following box.

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act. (check one)

Large accelerated filer

Accelerated filer

Non-accelerated filer   
(Do not check if a  
smaller reporting company)

Smaller reporting company   
Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided to Section 7(a)(2)(B) of the Securities Act.

**CALCULATION OF REGISTRATION FEE**

Title of Each Class of Securities to be Registered	Proposed Maximum Aggregate Offering Price(1)(2)	Amount of Registration Fee(3)
Common Stock, \$0.0001 par value per share	\$	\$

- (1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.
- (2) Includes the aggregate offering price of additional shares that the underwriters have the option to purchase.
- (3) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum aggregate offering price.

**The registrant hereby amends this registration statement on such date or dates as may be necessary to delay its effective date until the registrant shall file a further amendment which specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.**

The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

Subject to Completion  
Preliminary Prospectus dated \_\_\_\_\_, 2018

PROSPECTUS

Shares



Common Stock

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This is Y-mAbs Therapeutics, Inc.'s initial public offering. We are selling \_\_\_\_\_ shares of our common stock.

We expect the public offering price to be between \$ \_\_\_\_\_ and \$ \_\_\_\_\_ per share. Currently, no public market exists for the shares. After pricing of the offering, we expect that the shares will trade on the Nasdaq Global Market under the symbol "YMAB."

**We are an "emerging growth company" under federal securities laws and are subject to reduced public company disclosure standards. See "Summary—Implications of Being an Emerging Growth Company."**

**Investing in the common stock involves risks that are described in the "Risk Factors" section beginning on page 13 of this prospectus.**

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	<u>Per Share</u>	<u>Total</u>
Public offering price	\$ _____	\$ _____
Underwriting discount(1)	\$ _____	\$ _____
Proceeds, before expenses, to us	\$ _____	\$ _____

(1) We refer you to "Underwriting" beginning on page 202 for additional information regarding underwriting compensation.

The underwriters may also exercise their option to purchase up to an additional \_\_\_\_\_ shares from us, at the public offering price, less the underwriting discount, for 30 days after the date of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The shares will be ready for delivery on or about \_\_\_\_\_, 2018.

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*Joint Book-Running Managers*

**BofA Merrill Lynch**

**Cowen**

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The date of this prospectus is \_\_\_\_\_, 2018.

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**TABLE OF CONTENTS**

	<u>Page</u>
<a href="#">FOR INVESTORS OUTSIDE THE UNITED STATES</a>	<a href="#">ii</a>
<a href="#">PRESENTATION OF FINANCIAL INFORMATION</a>	<a href="#">ii</a>
<a href="#">SUMMARY</a>	<a href="#">1</a>
<a href="#">THE OFFERING</a>	<a href="#">9</a>
<a href="#">SUMMARY CONSOLIDATED FINANCIAL DATA</a>	<a href="#">11</a>
<a href="#">RISK FACTORS</a>	<a href="#">13</a>
<a href="#">SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA</a>	<a href="#">74</a>
<a href="#">USE OF PROCEEDS</a>	<a href="#">76</a>
<a href="#">DIVIDEND POLICY</a>	<a href="#">78</a>
<a href="#">CAPITALIZATION</a>	<a href="#">79</a>
<a href="#">DILUTION</a>	<a href="#">81</a>
<a href="#">SELECTED CONSOLIDATED FINANCIAL DATA</a>	<a href="#">84</a>
<a href="#">MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</a>	<a href="#">85</a>
<a href="#">BUSINESS</a>	<a href="#">100</a>
<a href="#">MANAGEMENT</a>	<a href="#">159</a>
<a href="#">EXECUTIVE AND DIRECTOR COMPENSATION</a>	<a href="#">167</a>
<a href="#">CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS</a>	<a href="#">179</a>
<a href="#">PRINCIPAL STOCKHOLDERS</a>	<a href="#">186</a>
<a href="#">DESCRIPTION OF CAPITAL STOCK</a>	<a href="#">189</a>
<a href="#">SHARES ELIGIBLE FOR FUTURE SALE</a>	<a href="#">194</a>
<a href="#">MATERIAL U.S. FEDERAL INCOME AND ESTATE TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK</a>	<a href="#">197</a>
<a href="#">UNDERWRITING</a>	<a href="#">202</a>
<a href="#">LEGAL MATTERS</a>	<a href="#">210</a>
<a href="#">EXPERTS</a>	<a href="#">210</a>
<a href="#">WHERE YOU CAN FIND ADDITIONAL INFORMATION</a>	<a href="#">211</a>
<a href="#">INDEX TO CONSOLIDATED FINANCIAL STATEMENTS</a>	<a href="#">F-1</a>

Neither we nor the underwriters have authorized anyone to provide you with any information or to make any representations other than those contained in this prospectus, any amendment or supplement to this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. Neither we nor the underwriters take responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front cover of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties or us. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. All of the market data used in this prospectus involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. While we believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data. The industry in which we operate is subject to a high degree

of uncertainty, change and risk due to a variety of factors, including those described in the section titled "Risk Factors." These and other factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.

### **FOR INVESTORS OUTSIDE THE UNITED STATES**

We have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

### **PRESENTATION OF FINANCIAL INFORMATION**

Pursuant to the applicable provisions of the Fixing America's Surface Transportation Act, we are not required to file our financial information for the historical 2015 annual period or for any interim period for 2016 or 2017 because we plan to file our financial information for the year ended December 31, 2017 in the first public filing of our registration statement. While the 2015 annual financial information and 2016 and 2017 interim financial information is otherwise required by Regulation S-X, we believe that it will not be required to be included in our registration statement at the time of the first public filing.

## SUMMARY

*This summary highlights, and is qualified in its entirety by, the more detailed information and financial statements included elsewhere in this prospectus. This summary does not contain all of the information that may be important to you in making your investment decision. You should read and carefully consider this entire prospectus, including the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes included elsewhere in this prospectus, before deciding to invest in our common stock.*

*Except as otherwise indicated herein or as the context otherwise requires, references in this prospectus to "Y-mAbs," the "company," "we," "us" and "our" and similar words refer to Y-mAbs Therapeutics, Inc. and our wholly owned Danish subsidiary, Y-mAbs Therapeutics A/S.*

### Overview

We are a late-stage clinical biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer. We have a broad and advanced product pipeline, including two pivotal-stage product candidates—naxitamab and omburtamab—which target tumors that express GD2 and B7-H3, respectively. We are developing naxitamab for the treatment of pediatric patients with relapsed or refractory, or R/R, high-risk neuroblastoma, or NB, and radiolabeled omburtamab for the treatment of pediatric patients with central nervous system, or CNS, leptomeningeal metastases, or LM, from NB. NB is a rare and almost exclusively pediatric cancer that develops in the sympathetic nervous system and CNS/LM is a rare and usually fatal complication of NB in which the disease spreads to the membranes, or meninges, surrounding the brain and spinal cord in the CNS.

We expect to submit a Biologics License Application, or BLA, for each of our two lead product candidates in 2018, with a goal of receiving approval by the U.S. Food and Drug Administration, or FDA, in 2019. We plan to commercialize both of our lead product candidates in the United States as soon as possible after obtaining FDA approval, if such approval occurs. We have two additional omburtamab follow-on product candidates in pre-clinical development, omburtamab-DTPA and hu-omburtamab, each targeting indications with large adult patient populations. We are also advancing an early-stage, novel pipeline of bispecific antibodies, or BsAbs. We believe our BsAbs have the potential to result in improved tumor-binding, longer serum half-life, lower immunogenicity and significantly greater T-cell mediated killing of tumor cells without the need for continuous infusion. Our mission is to become the world leader in developing better and safer antibody-based pediatric oncology products addressing clear unmet medical needs and, as such, have a transformational impact on the lives of patients. We intend to advance and expand our product pipeline into certain adult cancer indications either independently or in collaboration with potential partners.

Naxitamab is a recombinant humanized immunoglobulin G, or IgG1k, monoclonal antibody that targets ganglioside GD2, which is highly expressed in various neuroectoderm-derived tumors and sarcomas. Naxitamab is currently being studied in several clinical trials, including pivotal-stage development (Study 201) and a Phase 1/2 clinical trial (Study 12-230) for the treatment of pediatric R/R high-risk NB, a Phase 2 clinical trial (Study 16-1643) in front-line NB and a Phase 2 clinical trial (Study 15-096) for relapsed osteosarcoma. We believe that naxitamab has multiple potential advantages over other GD2-targeting antibody-based therapies. In particular, its modest toxicity allows for doses two-and-a-half times greater than existing GD2-targeting antibody-based therapies. Unlike currently approved GD2-targeting therapies for NB, which require 10 to 20 hours of infusion and hospitalization for several days, naxitamab is administered in approximately 30 minutes in an outpatient setting. We believe this significantly shorter administration time is an important advantage considering the overall pain associated with treatment.

In Study 12-230 for naxitamab, which together with Study 201 is expected to form the primary basis of our BLA submission, we achieved an overall response rate, or ORR, of 57% in 23 patients with pediatric R/R high-risk NB who at study entry had evaluable tumors and no evidence of progression of disease, or PD. Based on our discussions with the FDA, the profile of the non-PD R/R high-risk NB pediatric patients in Study 12-230 is representative of the intended patient population for naxitamab's target indication. The corresponding ORRs will form the primary objective of our pivotal study (Study 201). Additionally, based on our discussions with the FDA, we believe that naxitamab may qualify for accelerated approval if we can demonstrate a 30% ORR (which is significantly different from a 20% ORR at a 95% confidence interval, or CI) with a minimum 12-week duration of response. We have proposed to the FDA that, pending comparability between the study population and the pharmacokinetics analysis in Study 12-230 and Study 201, the data from the two studies may be pooled for analysis. Naxitamab has been administered to more than 200 patients to date, who will form the safety portion of our planned BLA submission, and has been generally well tolerated with no significant long-term toxicities observed to date. We expect to report the topline results from our ongoing Study 201 and submit the BLA for naxitamab for R/R high-risk NB in the second half of 2018. Currently, there are no FDA-approved therapies for primary refractory or second-line pediatric NB patients. Naxitamab has also received orphan drug designation, or ODD, and rare pediatric disease designation, or RPDD, from the FDA for the treatment of NB. While our current clinical efforts for naxitamab are focused on rare pediatric cancers, we believe that we can potentially expand its application to the treatment of adults with cancers that express GD2. We estimate that there were more than 200,000 new adult patients diagnosed with GD2-positive cancers in the United States in 2017.

Omburtamab is a murine monoclonal antibody that targets B7-H3, an immune checkpoint molecule that is widely expressed in tumor cells of several cancer types. <sup>131</sup>I-omburtamab, which is omburtamab radiolabeled with Iodine-131, is currently being studied in several clinical trials including pivotal-stage development (Study 101) and a Phase 1 clinical trial (Study 03-133) for the treatment of pediatric patients with R/R NB who have CNS or LM. As of August 2017, 93 patients with pediatric CNS/LM from NB had been treated with <sup>131</sup>I-omburtamab in Study 03-133. An analysis of these 93 patients demonstrated a median overall survival, or OS, of 47 months (including an estimated five-year OS of approximately 43%), as compared to historical median OS of approximately six months. We have proposed to the FDA that, pending comparability between study population and the pharmacokinetics analysis in Study 03-133 and Study 101, data from both studies may be pooled for analysis for our planned BLA submission. Omburtamab has also been generally well tolerated, with no significant long-term toxicities observed to date. <sup>131</sup>I-omburtamab has received ODD and RPDD from the FDA for the treatment of NB, and Breakthrough Therapy Designation, or BTD, for the treatment of pediatric patients with R/R NB who have CNS or LM. We expect to submit the BLA for <sup>131</sup>I-omburtamab for CNS/LM from NB in the second half of 2018.

<sup>124</sup>I-omburtamab, which is omburtamab radiolabeled with Iodine-124, is currently being studied for the treatment of Diffuse Intrinsic Pontine Glioma, or DIPG. <sup>131</sup>I-omburtamab is currently being studied for the treatment of Desmoplastic Small Round Cell Tumors, or DSRCT. Both DIPG and DSRCT are rare, and often fatal, cancers. While our current clinical efforts are focused on rare pediatric cancers, we believe we can potentially expand omburtamab's application to the treatment of CNS/LM resulting from other adult and pediatric solid tumors expressing B7-H3 and the underlying solid systemic tumors. We estimate that, in the United States in 2017, there were more than 30,000 new patients diagnosed with cancer that has metastasized to the CNS/LM, of which the vast majority express B7-H3.

We have two additional product candidates in pre-clinical development, omburtamab-DTPA (diethylenetriamine pentaacetate), a Lutetium-177 conjugated antibody, and hu-omburtamab, a humanized version of omburtamab, each targeting indications with large adult patient populations

where we believe there is a significant unmet medical need. We are also advancing a pipeline of novel BsAbs through late pre-clinical development, including our naxitamab-BsAb product candidate for the treatment of refractory GD2-positive adult and pediatric solid tumors and our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. In pre-clinical studies, naxitamab-BsAb has demonstrated the potential for improved tumor-binding, longer serum half-life, lower immunogenicity and significantly greater T-cell mediated killing compared to existing bispecific constructs. We expect to file an Investigational New Drug application, or IND, for our naxitamab-BsAb product candidate for treatment of patients with refractory GD2-positive solid tumors in the first half of 2018.

We have exclusive rights to MSK's rights in all of our current product candidates under our 2015 license agreement, or the MSK License, with Memorial Sloan Kettering Cancer Center, or MSK. The MSK License also provides us with non-exclusive access to technology that involves the creation of a novel human protein tag that can potentially dimerize, or link together, bispecific T-cell engagers, or BiTEs. We refer to this technology as the MULTI-TAG technology. We plan to create a broad platform of dimerized BiTEs using the MULTI-TAG technology and are currently collaborating with MSK on several MULTI-TAG product candidates, with the goal of selecting a potential clinical candidate in 2018. We believe that our strong relationship with MSK, one of the world's leading cancer treatment centers, and our access to certain of MSK's technologies and substantial research capabilities affords us several competitive advantages, particularly with respect to patient recruitment for clinical trials. Under a separate 2017 CD33 license agreement with MSK, or the MSK CD33 License, we have a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make, and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments in connection with certain CD33 antibodies developed in the laboratory of a specific principal investigator at MSK and constructs thereof.

Our management team has substantial public company experience and extensive knowledge in the field of antibody oncology drug development, manufacturing and commercialization. Thomas Gad, our Founder, Chairman, President and Head of Business Development, co-founded Singad Pharma ApS, a Danish pharmaceutical and distribution company, where, as part of senior management, he gained more than 12 years of experience in the pharmaceutical industry, including in business development, financing and licensing negotiations and manufacturing site qualification. Our Chief Executive Officer, Dr. Claus Juan Møller San Pedro, co-founded Genmab A/S, or Genmab, one of the largest public biotechnology companies in Europe, where he served as Executive Vice President and Chief Operating Officer for over 10 years. Our Chief Financial Officer, Bo Kruse, served as Genmab's Chief Financial Officer and was directly involved in several of Genmab's financing rounds including Genmab's initial public offering. In addition, since our inception in April 2015, we have raised approximately \$120 million from our founding investors and prominent biotechnology institutional investors, including HBM Healthcare Investments (Cayman) Ltd. and funds advised by or affiliated with Scopia Capital Management LP and Sofinnova Ventures, Inc., among others, and as of \_\_\_\_\_, 2018 we have cash and cash equivalents of \$ \_\_\_\_\_.



## Our Pipeline

The following table sets forth our product candidates and their current development stages, estimated development timelines and anticipated milestones.

Product Candidate	Target	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Next Anticipated Milestone(s)
Naxitamab	GD2	Relapsed (Second-Line) / Refractory High-Risk Neuroblastoma (Pediatric)	Ongoing pivotal trial <sup>1</sup>				<ul style="list-style-type: none"> <li>• 2H 2018 – Announce Phase 3 topline data</li> <li>• 2H 2018 – BLA submission</li> <li>• 2019 – Potential FDA approval</li> </ul>
		(Front-Line) Consolidation of 1 <sup>st</sup> Remission High-Risk Neuroblastoma (Pediatric)	Ongoing Phase 2 trial				<ul style="list-style-type: none"> <li>• 2019 – Announce topline data</li> </ul>
		Relapsed (Second-Line) Osteosarcoma <sup>2</sup>	Ongoing Phase 2 trial				<ul style="list-style-type: none"> <li>• 2019 – Announce topline data</li> </ul>
Omburtamab	B7-H3	CNS / Leptomeningeal Metastases from Neuroblastoma (Pediatric) ( <sup>125</sup> I) <sup>3</sup>	Ongoing pivotal trial <sup>4</sup>				<ul style="list-style-type: none"> <li>• 2H 2018 – BLA submission</li> <li>• 2019 – Potential FDA approval</li> </ul>
		Diffuse Intrinsic Pontine Glioma (Pediatric) ( <sup>125</sup> I) <sup>3</sup>	Ongoing Phase 1 trial				<ul style="list-style-type: none"> <li>• 1H 2018 – Announce Phase 1 topline data</li> </ul>
		Desmoplastic Small Round Cell Tumor (Pediatric) ( <sup>125</sup> I) <sup>3</sup>	Ongoing Phase 1 trial				<ul style="list-style-type: none"> <li>• 1H 2018 – Announce Phase 1 topline data</li> </ul>
Omburtamab-DTPA <sup>5</sup>	B7-H3	B7-H3 Positive CNS / Leptomeningeal Solid Tumors					<ul style="list-style-type: none"> <li>• Late 2018 / Early 2019 – Submit IND in B7-H3 Positive LM tumors</li> </ul>
hu-Omburtamab	B7-H3	Systemic Solid Tumors (Adult) (Third-Line)					<ul style="list-style-type: none"> <li>• 2019 – Submit IND</li> </ul>
Naxitamab-BsAb	GD2xCD3	Refractory GD2-Positive Solid Tumors					<ul style="list-style-type: none"> <li>• 1H 2018 – Submit IND</li> </ul>
huCD33-BsAb	CD33xCD3	Hematological Cancers Expressing CD33					<ul style="list-style-type: none"> <li>• 2019 – Submit IND</li> </ul>

- (1) Pivotal registration study supportive of a BLA submission to the FDA, comprised of Study 12-230 measuring pharmacokinetic, toxicity and efficacy and an additional Phase 3 study, Study 201, to prove comparability between study sites using a cGMP commercial manufacturer and that has also been designed to satisfy the confirmatory study and post-marketing requirements by the FDA.
- (2) Initial study represents pediatric and young adult patients.
- (3) Represents the radioactive isotope of iodine used to radiolabel omburtamab.
- (4) Pivotal registration study supportive of a BLA submission to the FDA, comprised of Study 03-133 measuring pharmacokinetic, toxicity and efficacy and an additional Phase 3 study, Study 101, to prove comparability between study sites using a cGMP commercial manufacturer and that has also been designed to satisfy the confirmatory study and post-marketing requirements by the FDA.
- (5) Omburtamab-DTPA is a DTPA-conjugated omburtamab labeled with Lutetium-177 as chelator.

## Our Strategy

Key elements of our strategy are:

- *Rapidly and concurrently advance our lead product candidates to regulatory approval.*
- *Expand the indications and target patient populations for our existing product candidates.*
- *Independently commercialize our product candidates in indications and territories where we believe we can maximize their value.*
- *Advance our novel BsAb product candidates that we believe may offer potential substantial benefits over existing bispecific constructs.*
- *Leverage our relationships with leading academic and clinical institutions to develop additional product candidates.*

### **Risks Associated with Our Business**

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus immediately following this prospectus summary. These risks include the following:

- We have a limited operating history and have incurred significant losses since our inception. We have no products approved for commercial sale and expect to incur significant losses for the foreseeable future and may never achieve or maintain profitability.
- We have never obtained marketing approval for a product candidate and we may be unable to obtain, or may be delayed in obtaining, marketing approval for any of our product candidates.
- Our payment obligations to MSK may be a drain on our cash resources, or may cause us to incur debt obligations or issue additional equity securities to satisfy such payment obligations.
- We will need substantial additional funding for our product candidates. If we fail to obtain additional funding for our product candidates, we may be forced to delay, reduce or eliminate our research and drug development programs or future commercialization efforts and our licenses and other agreements may be terminated.
- Our product candidates and related technologies are novel approaches to cancer treatment that present significant challenges, and our ability to generate product revenue is dependent on the success of one or more of our lead product candidates, which will require additional clinical testing before we can seek regulatory approval and begin commercial sales.
- We may expend our resources to pursue a particular product candidate or indication and fail to capitalize on other product candidates or indications that may be more profitable or for which there is a greater likelihood of success.
- Drug development is a lengthy and expensive process, with an uncertain outcome. If clinical trials of our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs, experience delays in completing, or ultimately be unable to complete, the development of our product candidates or be unable to obtain marketing approval. We may encounter substantial delays in our clinical trials, or may not be able to conduct our trials on the timelines we expect.
- The market opportunities for our product candidates may be limited to those patients who are ineligible for or have failed prior treatments and may be small. If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.
- Our product candidates may cause serious adverse events, or SAEs, undesirable side effects or have other properties that could halt their clinical development, prevent, delay, or cause the withdrawal of their regulatory approval, limit their commercial potential, or result in significant negative consequences, including death of patients. If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability, or that of any of the potential future collaborators, to market the drug could be compromised.
- The outcome of pre-clinical studies and early clinical trials may not be predictive of the success of later clinical trials, interim results of a clinical trial do not necessarily predict final

results, and the results of our clinical trials may not satisfy the requirements of the FDA or comparable foreign regulatory authorities.

- Research and development of biopharmaceutical products is inherently risky. We may not be successful in our efforts to create a pipeline of product candidates and develop commercially successful products. If we fail to develop additional product candidates, our commercial opportunity will be limited.
- Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.
- We currently have no marketing and sales organization and have no experience in marketing products. We may not be successful in commercializing our product candidates if and when they are approved unless we are able to establish sales and marketing capabilities or enter into agreements with third parties to sell and market such approved products.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.
- We have entered into several agreements with MSK that are important to our business. We may also form or seek other collaborations or strategic alliances or enter into additional licensing arrangements in the future but may not realize the benefits of such collaborations or strategic alliances. If we are unable to enter into collaborations in the future, or if such collaborations are not successful, our business could be adversely affected.
- Third parties have sponsored and conducted all clinical trials of our lead product candidates so far, and our ability to influence the design and conduct of such clinical trials has been limited. To date, we have incurred significant expenses and are obligated to make significant payments in the future with respect to such clinical trials. We plan to assume control over the future clinical and regulatory development of such product candidates, including obtaining sponsorship of existing INDs or filing new company-sponsored INDs, which will entail substantial additional expenses and may be subject to delay. Any failure by a third party to meet its obligations with respect to the clinical and regulatory development of our product candidates may delay or impair our ability to obtain regulatory approval for our product candidates and result in liability for our company.
- Even if we complete the necessary pre-clinical studies and clinical trials, the FDA regulatory approval process is lengthy, time-consuming, and inherently unpredictable, and we or any of our collaborators may experience significant delays in the clinical development and regulatory approval, if any, for the commercialization of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or any of our collaborators, will obtain marketing approval to commercialize a product candidate.
- The European Medicines Agency, or the EMA, or comparable foreign regulatory authorities, may disagree with our regulatory plans, including our plans to seek accelerated approval, and we may fail to obtain regulatory approval of our product candidates, which would prevent our product candidates from being marketed abroad. Any approval we are granted for our product candidates in the United States would not assure approval of our product candidates in foreign jurisdictions.
- Reimbursement decisions by third-party payors may have an adverse effect on pricing and market acceptance. If there is not sufficient reimbursement for our products, it is less likely that our products will be widely used.

- Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our lead product candidates, if approved, or any of our other product candidates that may be approved in the future, which would adversely affect our revenue and results of operations.
- Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.
- We have a limited number of employees and depend heavily on our executive officers and consultants. Our future success depends on our ability to retain our senior management and other key executives and to attract, retain and motivate qualified personnel. The loss of their services could materially harm our business.
- It has been determined that we have material weaknesses in our internal control over financial reporting. If our remediation of these material weaknesses is not effective, or if we experience additional material weaknesses or otherwise fail to maintain an effective system of internal controls in the future, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock. In addition, because of our status as an emerging growth company, our independent registered public accounting firm is not required to provide an attestation report as to our internal control over financial reporting for the foreseeable future.

### **Implications of Being an Emerging Growth Company**

As a company with less than \$1.07 billion of revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, as amended, or the JOBS Act. We may remain an emerging growth company until the earlier of (i) the last day of the fiscal year following the fifth anniversary of the completion of this offering, (ii) the last day of the fiscal year in which we have more than \$1.07 billion in annual gross revenue, (iii) the last day of the fiscal year in which we are deemed to be a "large accelerated filer" as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, or the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded \$700.0 million as of the last business day of the second fiscal quarter of such fiscal year, or (iv) the date on which we issue more than \$1 billion of non-convertible debt securities during the prior three-year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure and other requirements that are applicable to other public companies that are not emerging growth companies. In particular, as an emerging growth company, in this prospectus, we (i) will have provided only two years of audited financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure, (ii) may avail ourselves of the exemption from the requirement to obtain an attestation and report from our auditors on the assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, (iii) have not included all of the executive compensation-related information that would be required if we were not an emerging growth company and (iv) we may not require stockholder non-binding advisory votes on executive compensation or golden parachute arrangements. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

We have chosen to opt out of the extended transition periods available to emerging growth companies under the JOBS Act for complying with new or revised accounting standards. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition periods for complying with new or revised accounting standards is irrevocable.

### **Our Corporate Information**

We were incorporated under the laws of the State of Delaware on April 30, 2015. Our executive offices are located at 750 Third Avenue, 9<sup>th</sup> Floor, New York, NY 10017 and our telephone number is (212) 874-9841. Our website address is [www.ymabs.com](http://www.ymabs.com). The information contained on, or that can be accessed through, our website is not a part of this prospectus and is not incorporated by reference herein. We have included our website address in this prospectus solely as an inactive textual reference.

"Y-mAbs" is our common law trademark. Any other trademarks or service marks of our company appearing in this prospectus are the property of Y-mAbs Therapeutics, Inc. All other trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto. We do not intend our use or display of other entities' trade names, trademarks or service marks to imply a relationship with, or endorsement or sponsorship of us by, any other entity.

## THE OFFERING

<b>Common stock offered by us</b>	shares.
<b>Common stock to be outstanding immediately following this offering</b>	shares.
<b>Option to purchase additional shares</b>	The underwriters have the option to purchase up to an additional shares of common stock from us, at the public offering price, less the underwriting discount. The underwriters may exercise this option at any time within 30 days from the date of this prospectus.
<b>Use of proceeds</b>	<p>We estimate that the net proceeds to us from our issuance and sale of shares of our common stock in this offering will be approximately \$ million, assuming an initial public offering price of \$ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise in full their option to purchase additional shares, we estimate that the net proceeds from this offering will be approximately \$ million.</p> <p>We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows: (i) to fund our ongoing pivotal stage development through regulatory submission, and other clinical development and expansion into new indications of one of our lead product candidates, naxitamab, (ii) to fund our ongoing pivotal stage development through regulatory submission, and other clinical development and expansion into new indications of another of our lead product candidates, omburtamab, (iii) to fund our ongoing clinical development of our omburtamab-DTPA product candidate, (iv) to fund our ongoing clinical development of our BsAb product candidates, (v) to fund additional pre-clinical research and clinical development activity related to our other product candidates and programs, and (iv) the remainder for working capital and other general corporate purposes, which may include funding for additional research, hiring additional personnel, capital and commercialization expenditures and the costs of operating as a public company. See "Use of Proceeds."</p>
<b>Risk factors</b>	You should carefully read the "Risk Factors" section of this prospectus and other information included in this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
<b>Dividend policy</b>	We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. We do not intend to pay cash dividends in respect of our common stock in the foreseeable future.
<b>Proposed Nasdaq Global Market symbol</b>	"YMAB."

The number of shares of our common stock to be outstanding after this offering is based on shares of our common stock outstanding as of December 31, 2017.

The number of shares of our common stock to be outstanding after this offering excludes:

- \_\_\_\_\_ shares of our common stock issuable upon the exercise of stock options outstanding as of December 31, 2017, at a weighted average exercise price of \$ \_\_\_\_\_ per share; and
- \_\_\_\_\_ shares of our common stock remaining available for future issuance as of December 31, 2017 under our Amended and Restated 2015 Equity Incentive Plan, or the 2015 Plan.

Unless otherwise indicated, all information in this prospectus assumes or gives effect to:

- no exercise of the outstanding options described above;
- no exercise by the underwriters of their option to purchase \_\_\_\_\_ additional shares of our common stock; and
- the adoption of our amended and restated certificate of incorporation and bylaws, both of which we intend to file immediately prior to the completion of this offering.

**SUMMARY CONSOLIDATED FINANCIAL DATA**

The following tables set forth our summary consolidated financial data for the periods indicated. We have derived the consolidated statements of operations data for the years ended December 31, 2016 and 2017 and the consolidated balance sheet data as of December 31, 2017 from our audited consolidated financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected for any future period. You should read the following summary consolidated financial data together with the more detailed information contained in "Selected Consolidated Financial Data," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and the related notes included elsewhere in this prospectus.

	Year Ended December 31,	
	2016	2017
	(in thousands, except per share data)	
<b>Consolidated Statement of Operations Data:</b>		
Revenue	\$	—
Operating expenses:		
Research and development		13,855
General and administrative		3,184
Total operating expenses		17,039
Loss from operations		(17,039)
Interest and other income (expense)		(18)
Net loss	\$	(17,057)
Net loss attributable to common stockholders	\$	(17,057)
Net loss per share attributable to common stockholders—basic and diluted <sup>(1)</sup>	\$	(1.21)
Weighted-average common shares outstanding used in computing net loss per share attributable to common stockholders—basic and diluted <sup>(1)</sup>		14,087,456
As adjusted net loss per share—basic and diluted (unaudited)		\$
As adjusted weighted average common shares outstanding used in computing net loss per share—basic and diluted (unaudited)		

- (1) See Note 4 to our consolidated financial statements included elsewhere in prospectus for an explanation of the method used to calculate the historical basic and diluted net loss per common share and the weighted average number of shares used in the computation of the per share amounts.



	As of December 31, 2017	
	Actual	As Adjusted(2)(3)
	(in thousands)	
<b>Consolidated Balance Sheet Data:</b>		
Cash and cash equivalents	\$	\$
Working capital <sup>(1)</sup>		
Total assets		
Total liabilities		
Accumulated deficit		
Total stockholders' equity		

(1) We define working capital as current assets less current liabilities.

(2) The as adjusted balance sheet data give effect to our issuance and sale of \_\_\_\_\_ shares of our common stock in this offering at an assumed initial public offering price of \$ \_\_\_\_\_ per share of common stock, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

(3) The as adjusted information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. A \$1.00 increase or decrease in the assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by \$ \_\_\_\_\_ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase or decrease of 1,000,000 shares of common stock in the number of shares offered by us, at an assumed initial public offering price of \$ \_\_\_\_\_ per share of common stock, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by \$ \_\_\_\_\_ million assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

## RISK FACTORS

*Investing in our common stock involves a high degree of risk. Before you decide to invest in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus, including "Management's Discussion and Analysis of Financial Condition and Results of Operation" and our consolidated financial statements and the related notes. The risks and uncertainties described below are the risks that we believe are material to us as of the date of this prospectus. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.*

### **Risks Related to Our Financial Condition and Need for Additional Capital**

***We have a limited operating history and have incurred significant losses since inception. We have no products approved for commercial sale and we expect to incur significant losses for the foreseeable future. We may never achieve or maintain profitability, which may cause the market value of our common stock to decline significantly.***

We are a clinical-stage biopharmaceutical company with a limited operating history. Since our inception in 2015, we have incurred significant losses each year. Our net loss was \$            million for the year ended December 31, 2017 and \$17,057 million for the year ended December 31, 2016. As of December 31, 2017, we had an accumulated deficit of \$            million. We have financed our operations principally through private placements of our common stock. To date, we have devoted substantially all of our efforts to research and development of our lead product candidates. While our lead product candidates are in pivotal clinical trials, we cannot assure you that we will receive regulatory approval for the sale of these or other product candidates in the near term, if at all. Our other product candidates are in the early stages of clinical development or pre-clinical research. As a result, we expect that it will be a number of years, if ever, before we have any of these other product candidates approved and ready for commercialization. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter.

We have no product candidates approved for commercial sale, have not generated any revenue from product sales, and do not anticipate generating any revenue from product sales until sometime after we receive regulatory approval for the commercial sale of a product candidate. We cannot assure you that we will ever receive regulatory approval for any of our product candidates.

Our ability to generate revenue and achieve profitability depends significantly on our success in many factors, including:

- completing research regarding, and non-clinical and clinical development of, our product candidates;
- obtaining regulatory approvals and marketing authorizations for product candidates for which we complete clinical studies;
- developing a sustainable and scalable manufacturing process for our product candidates, including establishing and maintaining commercially viable supply relationships with third parties and establishing our own manufacturing capabilities and infrastructure;
- launching and commercializing product candidates for which we obtain regulatory approvals and marketing authorizations, either directly or with a collaborator or distributor;
- obtaining market acceptance of our product candidates as viable treatment options;

- addressing any competing products, product candidates, related technologies and/or market developments;
- identifying, assessing, acquiring and/or developing new product candidates;
- negotiating favorable terms in any collaboration, licensing, or other arrangements into which we may enter;
- maintaining, protecting, and expanding our portfolio of intellectual property rights, including patents, trade secrets, and know-how; and
- attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring research, development, clinical trial, manufacturing and marketing costs associated with commercializing any approved products. Our expenses could increase beyond expectations if we are required by the FDA or other regulatory agencies, domestic or foreign, to change our manufacturing processes or assays, or to perform clinical, non-clinical, or other types of studies in addition to those that we currently anticipate. If we are successful in obtaining regulatory approvals to market one or more of our product candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain reimbursement at any price, and whether we own the commercial rights for that territory. If the number of our addressable disease patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the reasonably expected population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. If we are not able to generate sufficient revenue from the sale of any approved products, we may never become profitable.

***Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.***

We were incorporated and began our operations on April 30, 2015. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, conducting clinical trials of our lead product candidates, conducting pre-clinical studies of our other product candidates, and identifying additional potential product candidates. We have not yet demonstrated our ability to successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial-scale drug or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful drug commercialization. Typically, it takes about six to 10 years to develop a new drug from the time it is in Phase 1 clinical trials to when it is approved for treating patients, but in many cases it may take longer. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products. In addition, as a business with a limited operating history, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors as we continue to develop and commercialize our product candidates. As we continue to build our business, we expect our financial condition and operating results to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any particular quarterly or annual periods as indications of future operating performance.

***Our payment obligations to MSK may be a drain on our cash resources, or may cause us to incur debt obligations or issue additional equity securities to satisfy such payment obligations, which may adversely affect our financial position and results of operations.***

Under the MSK License, we have committed to funding scientific research as well as conducting certain clinical trial activities at MSK through 2020. As licensed product candidates progress through clinical development and commercialization, certain milestone payments will come due, and we will owe MSK customary royalties on commercial sales of our approved products, if any, including, unless such royalties become due earlier, an annual fixed minimum royalty of \$80,000 over the royalty term starting in 2020. These milestone payments become due upon achievement of the related clinical, regulatory or sales-based milestone set forth in the MSK License. Certain of the clinical and regulatory milestone payments become due at the earlier of completion of the related milestone activity or the date indicated in the MSK License, whether or not the milestone activity has been achieved. Total clinical and regulatory milestones potentially due under the MSK License are \$2,450,000 and \$9,000,000, respectively. There are also sales-based milestones that become due should we achieve certain amounts of sales of licensed products with total sales-based milestones potentially due of \$20,000,000. In addition, we have committed to acquire certain personnel and laboratory services at MSK under a Master Data Services Agreement, or MDSA, and two separate Core Facility Service Agreements, or CFSAs. We have also entered into an Investigator-Sponsored Master Clinical Trial Agreement, or MCTA, under which we will provide drug product and funding for certain clinical trials at MSK under separate appendices to be executed. Additionally, we entered into a master sponsored research agreement, or the SRA, with MSK pursuant to which we agreed to pay MSK to conduct certain research projects over a period of five years related to the intellectual property licensed under the MSK License.

These payments could be significant and in order to satisfy our obligations to MSK, if and when they are triggered, we may use our existing cash, incur debt obligations or issue additional equity securities, which may materially and adversely affect our financial position and results of operations.

***If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders if we issue equity securities, cause us to incur debt or assume contingent liabilities, and subject us to other risks.***

We may evaluate various acquisitions and strategic partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- the issuance of our equity securities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integration;
- the diversion of our management's attention from our existing product programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and

- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities, which could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

***We will need substantial additional funding for our product candidates. If we fail to obtain additional funding for our product candidates, we may be forced to delay, reduce or eliminate our research and drug development programs or future commercialization efforts and our license and other agreements may be terminated.***

Developing pharmaceutical products, including conducting pre-clinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We expect our expenses to increase in connection with our ongoing activities, particularly as we conduct clinical trials of, and seek marketing approval for our lead product candidates and our other product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur commercialization expenses, which may be significant, related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of any collaborator that we may have at such time for any such product candidate. Furthermore, commencing upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise sufficient amounts of additional capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate our research and drug development programs or our future commercialization efforts.

As of December 31, 2017, we had approximately \$            million in cash and cash equivalents. We estimate that the net proceeds from this offering will be approximately \$            million after deducting the estimated offering expenses payable by us. We believe that such proceeds, together with our existing cash and cash equivalents, will be sufficient to fund our operations through February 2020. However, changing circumstances may cause us to increase our spending significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We will require additional capital for further development and commercialization of our product candidates and may need to raise additional funds earlier if we choose to expand more rapidly than we presently anticipate.

In addition, we cannot be certain that additional funding will be available on acceptable terms, or at all. We have no firmly committed source of additional capital and if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or other research and development initiatives. Our licenses and other agreements may also be terminated if we are unable to meet the payment obligations under the agreements. We could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

***Raising additional capital may cause dilution to our stockholders, including purchasers of common stock in this offering, restrict our operations or require us to relinquish rights to our product candidates on terms unfavorable to us.***

We expect our expenses to increase in connection with our planned operations. Until such time, if ever, as we can generate substantial revenues from the sale of our product candidates, we expect to finance our cash needs through a combination of cash on hand, equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. In addition, debt financing, if available, would result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of our product candidates.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights related to our intellectual property, future revenue streams or any of our future product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, reduce and/or eliminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

***We may expend our resources to pursue a particular product candidate or indication and fail to capitalize on other product candidates or indications that may be more profitable or for which there is a greater likelihood of success.***

We intend to focus our efforts and managerial resources on specific product candidates and on specific indications. As a result, we may forgo or delay pursuit of opportunities with other product candidates or for other indications that may prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Failure to properly assess potential product candidates for indications could result in focusing on product candidates for indications with lower market potential, which could harm our business and financial condition. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through partnering, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate or product.

***It has been determined that we have material weaknesses in our internal control over financial reporting. If our remediation of these material weaknesses is not effective, or if we experience additional material weaknesses or otherwise fail to maintain an effective system of internal controls in the future, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock. In addition, because of our status as an emerging growth company, our independent registered public accounting firm is not required to provide an attestation report as to our internal control over financial reporting for the foreseeable future.***

Our management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles, or GAAP. As a result of becoming a public company, we will be required, pursuant to Section 404 of the Sarbanes-Oxley Act, to furnish a report by our management on, among other things, the effectiveness of our internal control over financial reporting for the first fiscal year beginning after the effective date of the registration statement of which this prospectus is a part. This assessment will need to include disclosures of any material weaknesses identified by our management in our internal control over financial reporting. A "material weakness" is a deficiency, or a combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. We are in the very early stages of the costly and challenging process of planning the activities necessary to perform the evaluation needed to comply with Section 404.

In connection with the audit of our financial statements for the year ended December 31, 2016, it was determined that we lack a sufficient number of trained professionals with an appropriate level of accounting knowledge, training and experience to: (a) design and maintain formal accounting policies, procedures and controls over the fair presentation of our financial statements; (b) analyze, record and disclose complex accounting matters timely and accurately, including share-based compensation arrangements and accounting for license arrangements; and (c) design and maintain controls over the preparation and review of account reconciliations, journal entries and financial statements, including maintaining appropriate segregation of duties.

Each of these control deficiencies could result in a misstatement of these accounts or disclosures that would result in a material misstatement of our annual or interim consolidated financial statements that would not be prevented or detected, and accordingly, it was determined that these control deficiencies constitute material weaknesses.

We have begun evaluating and implementing additional procedures to address these material weaknesses, however, we cannot assure you that these or other measures will fully remediate the material weaknesses described above in a timely manner. We intend to begin addressing the material weaknesses identified above by hiring additional finance and accounting personnel and increasing the oversight and review procedures with regard to financial reporting, financial processes and procedures and internal control procedures. Nevertheless, we cannot assure you that we will be able to remedy our current material weaknesses. If we are unable to remediate the material weaknesses, or otherwise maintain effective internal control over financial reporting, we may not be able to report our financial results accurately, prevent fraud or file our periodic reports in a timely manner. If our remediation of these material weaknesses is not effective, or if we experience additional material weaknesses or otherwise fail to maintain an effective system of internal controls in the future, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock. We cannot assure you that all of our existing material weaknesses have been identified, or that we will not in the future identify additional material weaknesses.

Our independent registered public accounting firm will not be required to formally attest to the effectiveness of our internal control over financial reporting pursuant to Section 404 until the later of the year following our first annual report required to be filed with the SEC, or the date we are no longer an "emerging growth company" as defined in the JOBS Act, if we take advantage (as we expect to do) of the exemptions contained in the JOBS Act. We will remain an "emerging growth company" for up to five years, although if the market value of our common stock that is held by non-affiliates exceeds \$700 million as of June 30 of any year before that time, we would cease to be an "emerging growth company" as of December 31 of that year. At such time, our independent registered public accounting firm may issue a report that is adverse in the event it is not satisfied with the level at which our controls are documented, designed or operating. Our remediation efforts may not enable us to avoid material weaknesses in our internal control over financial reporting in the future.

If we are unsuccessful in building an appropriate accounting infrastructure, we may not be able to prepare and disclose, in a timely manner, our financial statements and other required disclosures, or comply with existing or new reporting requirements. Any failure to report our financial results on an accurate and timely basis could result in sanctions, lawsuits, delisting of our shares from the Nasdaq Global Market or other adverse consequences that would materially harm to our business. If we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed and investors could lose confidence in our reported financial information. Any of the foregoing occurrences, should they come to pass, could negatively impact the public perception of our company, which could have a negative impact on our stock price.

#### **Risks Related to Product Development and Commercialization**

***Our product candidates and related technologies are novel approaches to cancer treatment that present significant challenges, and our ability to generate product revenue is dependent on the success of one or more of our lead product candidates, which will require additional clinical testing before we can seek regulatory approval and begin commercial sales.***

Our product candidates and related technologies represent novel approaches to cancer treatment generally, and developing and commercializing our product candidates subjects us to a number of challenges. We currently generate no revenues from sales of any products, we have never obtained marketing approval for a product candidate and we may never be able to develop a marketable product. Our ability to generate product revenue is highly dependent on our ability to obtain regulatory approval of and successfully commercialize one or more of our lead product candidates, which will require additional clinical and non-clinical development, regulatory review and approval in each jurisdiction in which we intend to market them, substantial investment, access to sufficient commercial manufacturing capacity, and significant marketing efforts before we can generate any revenue from product sales. We cannot be certain that any of our product candidates will be successful in clinical studies and they may not receive regulatory approval even if they are successful in clinical studies.

The success of our product candidates, including our lead product candidates, will depend on several factors, including the following:

- successful and timely completion of our ongoing clinical trials;
- initiation and successful patient enrollment and completion of additional clinical trials on a timely basis;
- safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority for marketing approval;
- timely receipt of marketing approvals for our lead product candidates from applicable regulatory authorities;



- the performance of our future collaborators, if any;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party raw materials and drug product suppliers and manufacturers;
- establishment of scaled production arrangements with third-party manufacturers to obtain finished products that are appropriately packaged for sale;
- obtaining and maintaining patent protection, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio, including our licensed intellectual property;
- successful launch of commercial sales following any marketing approval;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by patients, the medical community and third-party payors; and
- our ability to compete with other therapies.

We do not have complete control over many of these factors, including certain aspects of clinical development and the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing, distribution and sales efforts of any future collaborator.

In addition, because our lead product candidates are our most advanced product candidates, and because our other product candidates are based on similar technology, if our lead product candidates encounter safety or efficacy problems, developmental delays, regulatory issues, or other problems, our development plans and business could be significantly harmed. Further, competitors who are developing products with similar technology may experience problems with their products that could identify problems that would potentially harm our business.

***Drug development is a lengthy and expensive process, with an uncertain outcome. If clinical trials of our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs, experience delays in completing, or ultimately be unable to complete, the development of our product candidates or be unable to obtain marketing approval. We may encounter substantial delays in our clinical trials, or may not be able to conduct our trials on the timelines we expect.***

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must complete pre-clinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. Failure of one or more clinical trials can occur at any stage of testing. The outcome of pre-clinical studies and early-stage clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial, such as the results of our ongoing clinical trials of our lead product candidates, do not necessarily predict final results. Moreover, pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in pre-clinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs.

We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. In addition, we cannot be sure that we will be able to submit INDs for any of our product candidates in the future and we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin. Moreover, even if these trials begin, issues may arise that could suspend or terminate such clinical trials. A failure of one or more clinical studies can occur at any stage of testing, and our future clinical studies may not be successful.

***The market opportunities for our product candidates may be limited to those patients who are ineligible for or have failed prior treatments and may be small. If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.***

Our current potential patient population is based on our beliefs and estimates regarding the incidence or prevalence of certain types of cancers that may be addressable by our product candidates, which is derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, or market research. Our projections may prove to be incorrect and the number of potential patients may turn out to be lower than expected. Even if we obtain significant market share for our product candidates, because the potential target populations are small, we may never achieve profitability without obtaining regulatory approval for additional indications, including use of our product candidates for front-line and second-line therapy.

We expect to initially seek approval of some of our product candidates as second or third-line therapies for patients who have failed other approved treatments. Subsequently, for those product candidates that prove to be sufficiently beneficial, if any, we would expect to seek approval as a second-line therapy and potentially as a front-line therapy, but there is no guarantee that our product candidates, even if approved for third-line therapy, would be approved for second-line or front-line therapy. In addition, we may have to conduct additional clinical trials prior to gaining approval for second-line or front-line therapy.

The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the trial until its conclusion. We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons, including:

- the size and nature of the patient population;
- the patient eligibility criteria defined in the protocol;
- the size of the study population required for analysis of the trial's primary endpoints;
- the proximity of patients to trial sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- competing clinical trials for similar therapies or other new therapeutics not involving our product candidates and or related technologies;
- clinicians' and patients' perceptions as to the potential advantages and side effects of the product candidate being studied in relation to other available therapies, including any new drugs or treatments that may be approved for the indications we are investigating;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will not complete a clinical trial.

In addition, our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our trials may instead opt to enroll in a trial being conducted by one of our competitors. We expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials at such clinical trial sites. Moreover, because our product candidates represent a departure from more commonly used methods for cancer treatment, potential patients and their doctors may be inclined to only use conventional therapies, such as chemotherapy and radiation, rather than enroll patients in any future clinical trial.

Even if we are able to enroll a sufficient number of patients in our clinical trials, delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these trials and adversely affect our ability to advance the development of our product candidates.

***Our product candidates may cause serious adverse events, or SAEs, undesirable side effects or have other properties that could halt their clinical development, prevent, delay, or cause the withdrawal of their regulatory approval, limit their commercial potential, or result in significant negative consequences, including death of patients. If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability, or that of any potential future collaborators, to market the drug could be compromised.***

As with most biological drug products, use of our product candidates could be associated with undesirable side effects or adverse events which can vary in severity from minor reactions to death and in frequency from infrequent to prevalent. Undesirable side effects or unacceptable toxicities caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or halt clinical trials. To date, there have been no significant long-term toxicities among patients treated with our lead product candidates.

Treatment-related undesirable side effects or adverse events could also affect patient recruitment or the ability of enrolled subjects to complete the trial, or could result in potential product liability claims. In addition, these side effects may not be appropriately or timely recognized or managed by the treating medical staff, particularly outside of the research institutions that collaborate with us. We expect to have to educate and train medical personnel using our product candidates to understand their side effect profiles, both for our planned clinical trials and upon any commercialization of any product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in adverse effects to patients, including death. Any of these occurrences may materially and adversely harm our business, financial condition and prospects.

Clinical trials of our product candidates must be conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of any potential future collaborator, may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If one or more of our product candidates receives marketing approval and we, or others, discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, including during any long-term follow-up observation period recommended or required for patients who receive treatment using our products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product or seize the product;

- we, or any future collaborators, may be required to recall the product, change the way such product is administered to patients or conduct additional clinical trials;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular product;
- regulatory authorities may require additional warnings on the label, such as a "black box" warning or a contraindication, or impose distribution or use restrictions;
- we, or any future collaborators, may be required to create a Risk Evaluation and Mitigation Strategy, or REMS, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we, or any future collaborators, may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- we, or any future collaborators, could be sued and held liable for harm caused to patients;
- the drug may become less competitive; and
- our reputation may suffer.

Any of the foregoing could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations, and prospects, and could adversely impact our financial condition, results of operations or the market price of our common stock.

***The outcome of pre-clinical studies and early clinical trials may not be predictive of the success of later clinical trials, interim results of a clinical trial do not necessarily predict final results, and the results of our clinical trials may not satisfy the requirements of the FDA or comparable foreign regulatory authorities.***

Success in pre-clinical studies and early-stage clinical trials does not mean that future larger registration clinical trials will be successful because product candidates in later-stage clinical trials may fail to demonstrate sufficient safety and efficacy to the satisfaction of the FDA and non-U.S. regulatory authorities despite having progressed through pre-clinical studies and early-stage clinical trials. Product candidates that have shown promising results in pre-clinical studies and early-stage clinical trials may still suffer significant setbacks in subsequent clinical trials. Additionally, the outcome of pre-clinical studies and early-stage clinical trials may not be predictive of the success of later-stage clinical trials.

From time to time, we may publish or report interim or preliminary data from our clinical trials. Interim or preliminary data from clinical trials that we may conduct may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Interim or preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the interim or preliminary data. As a result, interim or preliminary data should be viewed with caution until the final data are available.

In addition, the design of a clinical trial can determine whether its results will support approval of a drug and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We may be unable to design and conduct a clinical trial to support marketing approval. Further, if our product candidates are found to be unsafe or lack efficacy, we will not be able to obtain marketing approval for them and our business would be harmed. A number of companies in the pharmaceutical industry, including those with greater resources and experience than us, have suffered significant setbacks in advanced clinical trials, even after obtaining promising results in pre-clinical studies and earlier clinical trials.

In some instances, there can be significant variability in safety and efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial protocols, differences in size and type of the patient populations, differences in and adherence to the dosing regimen and other trial protocols and the rate of dropout among clinical trial participants. We do not know whether any clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain marketing approval to market our product candidates. We have multiple clinical trials of our lead product candidates currently ongoing. In the event that an adverse safety issue, clinical hold or other adverse finding occurs in one or more of our clinical trials of our lead product candidates, such event could adversely affect our other clinical trials of our lead product candidates.

Further, our product candidates may not be approved even if they achieve their primary endpoints in Phase 3 trials or other pivotal trials. The FDA or non-U.S. regulatory authorities may disagree with our trial design and our interpretation of data from pre-clinical studies and clinical trials. In addition, any of these regulatory authorities may change requirements for the approval of a product candidate even after reviewing and providing comments or advice on a protocol for a pivotal clinical trial that has the potential to result in approval by the FDA or another regulatory authority. In addition, any of these regulatory authorities may also approve a product candidate for fewer or more limited indications than we request or may grant approval contingent on the performance of costly post-marketing clinical trials. The FDA or other non-U.S. regulatory authorities may not approve the labeling claims that we believe would be necessary or desirable for the successful commercialization of our product candidates.

Before obtaining marketing approvals for the commercial sale of any product candidate for a target indication, we must demonstrate with substantial evidence gathered in pre-clinical studies and well-controlled clinical studies, and, with respect to approval in the United States, to the satisfaction of the FDA, that the product candidate is safe and effective for use for that target indication. There is no assurance that the FDA or non-U.S. regulatory authorities will consider our future clinical trials to be sufficient to serve as the basis for approval of one of our product candidates for any indication. The FDA and non-U.S. regulatory authorities retain broad discretion in evaluating the results of our clinical trials and in determining whether the results demonstrate that a product candidate is safe and effective. If we are required to conduct additional clinical trials of a product candidate than we expect prior to its approval, we will need substantial additional funds and there is no assurance that the results of any such additional clinical trials will be sufficient for approval.

***Research and development of biopharmaceutical products is inherently risky. We may not be successful in our efforts to create a pipeline of product candidates and develop commercially successful products. If we fail to develop additional product candidates, our commercial opportunity will be limited.***

The product candidates and related technologies we have licensed have not yet led, and may never lead, to approved or commercially successful products. Even if we are successful in continuing to build our pipeline, obtaining regulatory approvals and commercializing our product candidates will require substantial additional funding beyond the net proceeds of this offering and are prone to the risks of failure inherent in medical product development. Investment in biopharmaceutical product development involves significant risk that any potential product candidate will fail to demonstrate adequate efficacy or an acceptable safety profile, gain regulatory approval, and/or become commercially viable. We cannot provide you any assurance that we will be able to successfully advance any of these additional product candidates through the development process. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development or commercialization for many reasons, including the following:

- we may not be successful in identifying additional product candidates;

- we may not be able to assemble sufficient resources to acquire or discover additional product candidates;
- our product candidates may not succeed in pre-clinical or clinical testing;
- a product candidate may on further study be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may nevertheless be covered by third parties' patents or other exclusive rights;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community or third-party payors, if applicable.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, discover, develop, or commercialize additional product candidates, which would have a material adverse effect on our business and could potentially cause us to cease operations. Even if we receive approval to market our product candidates from the FDA, the EMA, or other regulatory bodies, whether for the treatment of cancers or other diseases, we cannot assure you that any such product candidates will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives.

***Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.***

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. For example, current cancer treatments like chemotherapy and radiation therapy are well-established in the medical community, and doctors may continue to rely on these treatments. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant revenues from sales of drugs and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- developing processes for the safe administration of our products, including long-term follow-up for all patients who receive the product;
- the potential advantages of the product compared to competitive therapies;
- the prevalence and severity of any side effects;
- whether the product is designated under physician treatment guidelines as a first-, second- or third-line therapy;
- our ability, or the ability of any potential future collaborators, to offer the product for sale at competitive prices;

- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- limitations or warnings, including distribution or use restrictions contained in the product's approved labeling;
- the strength of sales, marketing and distribution support;
- changes in the standard of care for the targeted indications for the product; and
- availability and amount of coverage and reimbursement from government payors, managed care plans and other third-party payors.

***We currently have no marketing and sales organization and have no experience in marketing products. We may not be successful in commercializing our product candidates if and when they are approved unless we are able to establish sales and marketing capabilities or enter into agreements with third parties to sell and market such approved products.***

We do not have a sales or marketing infrastructure and have no experience in the sale or marketing of pharmaceutical drugs. We are not currently a party to a strategic collaboration that provides us with access to a collaborator's resources in selling or marketing drugs. To achieve commercial success for any approved drug for which sales and marketing is not the responsibility of any strategic collaborator that we may have in the future, we must either develop a sales and marketing organization or outsource these functions to other third parties. In the future, we may choose to build a sales and marketing infrastructure to market or co-promote some of our product candidates if and when they are approved, or enter into collaborations with respect to the sale and marketing of our product candidates.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training even a small sales force can be expensive and time-consuming and could delay any commercial launch of a product candidate. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our drugs on our own after obtaining any regulatory approval to gain market acceptance include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future drugs;
- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive drug lines;
- unforeseen costs and expenses associated with creating an independent sales and marketing organization; and
- inability to obtain sufficient coverage and reimbursement from third-party payors and governmental agencies.

If we enter into arrangements with third parties to perform sales and marketing services, our revenues from the sale of drugs or the profitability of these revenues to us are likely to be lower than if

we were to market and sell any drugs that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drugs effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

***A variety of risks associated with operating our business internationally could materially adversely affect our business.***

We plan to seek regulatory approval of our product candidates outside of the United States and, accordingly, we expect that we, and any potential collaborators in those jurisdictions, will be subject to additional risks related to operating in foreign countries, including:

- differing regulatory requirements in foreign countries;
- unexpected changes in tariffs, trade barriers, price and exchange controls, and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration, and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977, or FCPA, Office of Foreign Assets Control, or OFAC, Anti-Money Laundering Program as required by the Bank Secrecy Act and its implementing regulations, or comparable foreign laws;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our planned international operations may materially adversely affect our ability to attain or maintain profitable operations.

***We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.***

The biopharmaceutical industry, and the market for developing antibody-based products in particular, is characterized by intense competition and rapid innovation. Our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential



competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies, universities, and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations as well as established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized, or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our product candidates and related technologies.

Specifically, MacroGenics, Inc. and Daiichi Sankyo Co. are developing antibodies against B7-H3. In addition, United Therapeutics Corporation has commercialized Unituxin (dinutuximab), an antibody against GD2, in the United States and we believe EUSA Pharma (UK) Ltd. plans to commercialize a similar antibody (dinutuximab beta) under the name Isquette in Europe.

Even if we obtain regulatory approval of our product candidates, we may not be the first to market and that may affect the price or demand for our product candidates. Additionally, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances. Additionally, a competitor could obtain orphan product exclusivity from the FDA with respect to such competitor's product. If such competitor product is determined to be the same product as one of our product candidates, that may prevent us from obtaining approval from the FDA for such product candidate for the same indication for seven years, except in limited circumstances.

***We have entered into several agreements with MSK that are important to our business. We may also form or seek other collaborations or strategic alliances or enter into additional licensing arrangements in the future but may not realize the benefits of such collaborations or strategic alliances. If we are unable to enter into future collaborations, or if such collaborations are not successful, our business could be adversely affected.***

We currently have in place several agreements with MSK that are important and we may form or seek strategic alliances, create joint ventures or collaborations, or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to our product candidates and any future product candidates that we may develop. Any of these relationships may require us to incur other charges, increase our near and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy.

Further, arrangements with third parties, such as our arrangement with MSK, or any potential future collaborations we may enter into involving our product candidates, are subject to numerous risks, including the following:

- such third parties or any potential future collaborators may have significant discretion in determining the efforts and resources that they will apply to a collaboration;
- such third parties or any potential future collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- such third parties or any potential future collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial, abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- such third parties or any potential future collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates;
- product candidates discovered through such arrangements or any potential future collaborations with us may be viewed by such third parties or any potential future collaborators as competitive with their own product candidates or products, which may cause such third parties or collaborators to cease to devote resources to the commercialization of our product candidates;
- such third party or any potential future collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to their marketing and distribution;
- such third parties or any potential future collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and such third party or any potential future collaborator that cause the delay or termination of the research, development or commercialization of our product candidates, or that result in costly litigation or arbitration that diverts management attention and resources;
- such third parties or any potential future collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- such arrangements or any potential future collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates; and
- such third parties or any potential future collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to commercialize such intellectual property.

As a result, if we are unable to maintain current arrangements or enter into and maintain future arrangements and collaborations, or if such arrangements or collaborations are not successful,

our business could be adversely affected. If we enter into certain arrangements or collaboration agreements and strategic partnerships or license our products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture, which could delay our timelines or otherwise adversely affect our business. We also cannot be certain that, following a strategic transaction or license, we will achieve the revenue or specific net income that justifies such transaction. Any delays in entering into new collaborations or strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain territories for certain indications, which would harm our business prospects, financial condition, and results of operations.

***If we or third parties, such as CROs or CMOs, use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.***

Our research and development activities may involve the controlled use of potentially hazardous substances, including chemical and biological materials, by us or third parties, such as CROs and CMOs. The use of Iodine-131, Iodine-124 and Lutetium-177-labeled antibody treatments involves the inherent risk of exposure from gamma ray emissions, which can alter or harm healthy cells in the body. We and such third parties are subject to federal, state, and local laws and regulations in the United States and Europe governing the use, manufacture, storage, handling, and disposal of medical and hazardous materials. Although we believe that our and such third-parties' procedures for using, handling, storing, and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state, or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition, or results of operations. We currently maintain insurance coverage for injuries resulting from the hazardous materials we use; however, future claims may exceed the amount of our coverage. Also, we do not have insurance coverage for pollution cleanup and removal. Currently the costs of complying with such federal, state, local and foreign environmental regulations are not significant, and consist primarily of waste disposal expenses. However, they could become expensive, and current or future environmental laws or regulations may impair our research, development, production and commercialization efforts.

***Our internal computer systems, or those used by our third-party research institution collaborators, CROs or other contractors or consultants, may fail or suffer security breaches.***

Despite the implementation of security measures, our internal computer systems and those of our future CROs and other contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. Although to our knowledge we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we rely on our third-party research institution collaborators for research and development of our product candidates and other third parties for the manufacture of our product candidates and to conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

***Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.***

Our operations, and those of our third-party research institution collaborators, CROs, CMOs, suppliers, and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics, terrorist activities, and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. In addition, we rely on our third-party research institution collaborators for conducting research and development of our product candidates, and they may be affected by government shutdowns or withdrawn funding. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce and process our product candidates. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. Damage or extended periods of interruption to our third-party collaborators', including MSK's, corporate, development or research facilities due to fire, natural disaster, power loss, communications failure, unauthorized entry or other events could cause us to cease or delay development of some or all of our product candidates. Although we intend to maintain property damage and business interruption insurance coverage on these facilities, our insurance might not cover all losses under such circumstances and our business may be seriously harmed by such delays and interruption.

***If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.***

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our products;
- injury to our reputation;
- withdrawal of clinical trial participants and inability to continue clinical trials;
- initiation of investigations by regulators;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize any product candidate;
- loss of any potential future revenue; and

- a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. Although we carry \$5.0 million of clinical trial insurance, the amount of such insurance coverage may not be adequate, we may be unable to maintain such insurance, or we may not be able to obtain additional or replacement insurance at a reasonable cost, if at all. Our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

### **Risks Related to Our Dependence on Third Parties**

***Third parties have sponsored all clinical trials of our lead product candidates so far, and our ability to influence the design and conduct of such clinical trials has been limited. We have incurred significant expenses and are obligated to make significant payments in the future with respect to such clinical trials. We plan to assume control over the future clinical and regulatory development of such product candidates, including obtaining sponsorship of existing INDs or filing new company-sponsored INDs, which will entail substantial additional expenses and may be subject to delay. Any failure by a third party to meet its obligations with respect to the clinical and regulatory development of our product candidates may delay or impair our ability to obtain regulatory approval for our product candidates and result in liability for our company.***

We have sponsored only a limited number of clinical trials relating to our lead product candidates. Instead, faculty members at our third-party research institution collaborators, or those institutions themselves, have sponsored most of the clinical trials relating to these product candidates, in each case, under their own INDs. We have incurred significant expenses and are obligated to make significant payments in the future with respect to such clinical trials. To date, we have assumed control of only a limited number of such clinical trials and plan to assume control of the overall clinical and regulatory development of our lead product candidates for future clinical trials and obtain sponsorship of the INDs or file new company-sponsored INDs, all of which will cause us to incur substantial additional expenses and may be subject to delay. Failure to obtain, or delays in obtaining, sponsorship of INDs or in filing new company-sponsored INDs for these or any other product candidates we determine to advance could negatively affect the timing of our potential future clinical trials. Such an impact on timing could increase research and development costs and could delay or prevent obtaining regulatory approval for our most advanced product candidates, either of which could have a material adverse effect on our business.

Further, even in the event that the IND sponsorship is obtained for existing and new INDs, we did not control the design or conduct of the previous trials. It is possible that the FDA will not accept these previous trials as providing adequate support for future clinical trials, whether controlled by us or third parties, for any reason, including the safety, purity, and potency of the product candidate, the degree of product characterization, elements of the design or execution of the previous trials or safety concerns, or other trial results. We may also be subject to liabilities arising from any treatment-related injuries or adverse effects in patients enrolled in these previous trials. As a result, we may be subject to unforeseen third-party claims and delays in our potential future clinical trials. We may also be required to repeat in whole or in part clinical trials previously conducted by our third-party research institution collaborators, which will be expensive and delay the submission and licensure or other regulatory approvals with respect to any of our product candidates. Any such delay or liability could have a material adverse effect on our business.

Moreover, we have so far been dependent on contractual arrangements with our third-party research institution collaborators and will continue to be until we assume control. Such arrangements provide us certain information rights with respect to the previous trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the previous trials. However, if the data prove to be inadequate compared to the first-hand knowledge we might have gained had the completed trials been corporate-sponsored trials, then our ability to design and conduct our planned corporate-sponsored clinical trials may be adversely affected. Additionally, the FDA may disagree with the sufficiency of our right to reference the pre-clinical, manufacturing, or clinical data generated by these prior investigator-sponsored trials, or our interpretation of pre-clinical, manufacturing, or clinical data from these clinical trials. Moreover, the FDA may require us to obtain and submit additional pre-clinical, clinical, manufacturing, clinical, toxicology or other in vivo or in vitro data before we may begin our planned trials and/or may not accept such additional data as adequate to begin our planned trials.

***We will rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval of or commercialize our product candidates.***

We will rely on third parties to conduct our clinical trials under agreements with MSK, universities, medical institutions, CROs, strategic partners, and others. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory, and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with current good clinical practices, or cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of trial sponsors, principal investigators, and trial sites. If we or any of these third parties fail to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional non-clinical or clinical trials before approving our marketing applications. We cannot be certain that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the cGCP regulations. In addition, our clinical trials must be conducted with biologic product produced under cGMP regulations and will require a large number of test patients. Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed, or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our product candidates. We may also rely on investigator-reported interim data in making business decisions. Independent review of the data could fail to confirm the investigator-reported interim data, which may lead to revisions in disclosed clinical trial results in the future. Any such revisions that reveal more negative data than previously disclosed investigator-reported interim data could have an adverse impact on our business prospects and the trading price of our common stock. Such revisions could also reduce investor confidence in investigator-reported interim data that we disclose in the future.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves additional cost and delays and requires management time and focus. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges in the future or that these challenges will not have a material adverse impact on our business, financial condition and prospects.

***We will rely on third parties to manufacture our product candidates for our pre-clinical studies, and in the case of our lead product candidates, our ongoing clinical trials, as well as any additional clinical trials of our other product candidates we may conduct. We also expect to rely on third parties for the manufacturing process of our product candidates, if approved. Our business could be harmed if those third parties fail to provide us with sufficient quantities of product supplies or product candidates, or fail to do so at acceptable quality levels or prices.***

We do not currently own any facility that may be used as a clinical-scale manufacturing and processing facility and we intend to rely on outside vendors to manufacture supplies and process our product candidates for pre-clinical studies and clinical trials under the guidance our management team. Our lead product candidates have only been manufactured or processed on a limited basis and we may not be able to continue doing so for any of our product candidates. Our manufacturing process may be more difficult or expensive than the approaches currently in use. We may make changes as we work to optimize the manufacturing process, and we cannot be sure that even minor changes in the process will not result in significantly different products that may not be as safe and effective as any product candidates deployed by our third-party research institution collaborators.

To date, we have obtained the active pharmaceutical ingredient, or API, of our lead product candidates from a limited number of third-party manufacturers. We have engaged a separate third-party manufacturer to conduct fill-and-finish and labeling services, as well as for the storage and distribution of our lead product candidates to clinical sites. We do not have a long-term supply agreement with any of these third-party manufacturers, and we purchase our required drug supplies on a purchase order basis.

We expect to rely on third-party manufacturers or third-party collaborators for the manufacture of our product candidates for commercial supply of any of our product candidates for which we or any of our potential future collaborators obtain marketing approval. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- the number of potential manufacturers is limited and the FDA must approve any new manufacturers. This approval would require new testing and current good manufacturing practices, or cGMP, compliance inspections by the FDA. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products;
- our third-party manufacturers might be unable to timely manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any;
- our third-party manufacturers may not be able to execute our manufacturing procedures and other logistical support requirements appropriately;
- our third-party manufacturers may not perform as agreed, according to our schedule or specifications, or at all, may not devote sufficient resources to our product candidates, may give greater priority to the supply of other products over our product candidates, or may not

remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store, and distribute our products;

- our third-party manufacturers are subject to ongoing periodic unannounced inspection by the FDA and corresponding state agencies to ensure strict compliance with cGMPs and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these and or any other applicable regulations and standards;
- we may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our products;
- our third-party manufacturers could breach, terminate or not renew their agreement with us at a time that is costly or inconvenient for us;
- clinical and, if approved, commercial supplies for the raw materials and components used to manufacture and process our product candidates, particularly those for which we have no other source or supplier, may not be available or may not be suitable or acceptable for use due to material or component defects;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales. Our third-party manufacturers and critical reagent suppliers may be subject to inclement weather, as well as natural or man-made disasters; and
- our third-party manufacturers may have unacceptable or inconsistent product quality success rates and yields.

Each of these risks could delay or prevent the completion of our clinical trials or the approval of any of our product candidates by the FDA, result in higher costs or adversely impact commercialization of our product candidates. In addition, we will rely on third parties to perform certain specification tests on our product candidates prior to delivery to patients. If these tests are not appropriately done and test data are not reliable, patients could be put at risk of serious harm and the FDA could place significant restrictions on our company until deficiencies are remedied.

The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the FDA pursuant to inspections that will be conducted after we submit our BLA to the FDA. We do not have complete control over all aspects of the manufacturing process of, and are dependent on, our contract manufacturing partners for compliance with cGMP regulations for manufacturing both active drug substances and finished drug products. Any product candidates that we may develop may compete with product candidates of other companies for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our drugs and harm our business and results of operations.



Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply of our lead product candidates and we only currently use a different single third-party manufacturer for fill-and-finish services for our lead product candidates. If our current contract manufacturers cannot perform as agreed, we may be required to replace those manufacturers. Although we believe that there may be potential alternative manufacturers who could manufacture our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement.

***We are and will continue to rely in significant part on outside scientists and their third-party research institutions for research and development and early clinical testing of our product candidates. These scientists and institutions may have other commitments or conflicts of interest, which could limit our access to their expertise and adversely affect the timing of our IND filings and our ability to conduct future planned clinical trials.***

We currently have no internal research and development capabilities and we have not and are not currently conducting any independent clinical trials. Therefore, we currently rely on third-party research institutions for both capabilities.

Currently, MSK is conducting clinical trials to address pediatric R/R high-risk NB and a clinical trial to address relapsed osteosarcoma using our naxitamab product candidate. We are also conducting a clinical trial at MSK for CNS/LM from NB and clinical trials for DIPG and DSRCT for our omburtamab product candidate. Under the terms of the MSK License, we are obligated to pay for the costs associated with these clinical trials.

We have agreed to fund certain research and development costs under both the MSK License and the MSK CD33 License. However, the research we have agreed to fund constitutes only a small portion of the overall research of MSK. Other research being conducted by MSK may receive higher priority than research on the programs we may fund.

The outside scientists who conduct the clinical testing of our current product candidates, and who conduct the research and development upon which our product candidate pipeline depends, are not our employees; rather they serve as either independent contractors or the primary investigators under research and other agreements that we have entered into with MSK. Such scientists and collaborators may have other commitments that would limit their availability to us. Although our scientific advisors generally agree not to do competing work, if an actual or potential conflict of interest between their work for us and their work for MSK or another entity arises, we may lose their services. These factors could adversely affect the timing of our IND filings and our ability to conduct future planned clinical trials. It is also possible that some of our valuable proprietary knowledge may become publicly known through these scientific advisors if they breach their confidentiality agreements with us, which would cause competitive harm to, and have a material adverse effect on, our business.

Our existing agreements with MSK may be subject to termination by MSK upon the occurrence of certain circumstances as described in more detail in the section of this prospectus captioned "Business—Intellectual Property—MSK License." If MSK terminates the MSK License, the MSK CD33 License or its other agreements with us, the research and development of the relevant product candidates would be suspended, and we would not be able to research, develop, and license our existing and future product candidates as currently contemplated. We may be required to devote additional resources to the development of our product candidates or seek a new collaboration partner, and the terms of any additional collaborations or other arrangements that we establish may not be favorable to us. Switching or adding third parties to conduct our clinical trial would involve substantial costs and delays and require extensive management time and focus, which can materially impact our ability to meet our desired clinical development timelines.

***Our product candidates are biologics and the manufacture of our product candidates is complex. We, or any of our third-party manufacturers, may encounter difficulties in production, particularly with respect to process development or scaling-up of our manufacturing capabilities. For some reagents, equipment, and materials, we rely or may rely on sole source vendors or a limited number of vendors. Such difficulties may result in an inadequate supply of our product candidates for clinical trials or our products for patients, if approved, could be delayed or stopped, or we may be unable to maintain a commercially viable cost structure.***

Our product candidates are biologics and the process of manufacturing them is complex, highly-regulated and subject to multiple risks. As a result of the complexities, the cost to manufacture biologics is generally higher than traditional small molecule chemical compounds, and the manufacturing process for biologics is less reliable and is more difficult to reproduce. In addition, manufacturing our product candidates will require many reagents, which are substances used in our manufacturing processes to bring about chemical or biological reactions, and other specialty materials and equipment, some of which are manufactured or supplied by small companies with limited resources and experience to support commercial biologics production. Our manufacturing process may be susceptible to product loss or failure due to interruptions in the manufacturing process variability in product characteristics, quality control, contamination, equipment or reagent failure, improper installation or operation of equipment, product testing, vendor or operator error, availability of qualified personnel, logistics and shipping as well as compliance with strictly enforced federal, state and foreign regulations. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects, and other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot assure you that any stability failures or other issues relating to the manufacture of our product candidates will not occur in the future.

Further, as product candidates are developed through pre-clinical to late-stage clinical trials towards approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods, are altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives, and any of these changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials. Moreover, as we develop and/or scale-up our manufacturing process, we expect that we will need to obtain rights to and supplies of certain materials and equipment to be used as part of that process. We may not be able to obtain rights to such materials on commercially reasonable terms, or at all.

In addition, the manufacturing process for any products that we may develop is subject to FDA, EMA and other foreign regulatory authority approval process, and we will need to contract with manufacturers who can meet all applicable FDA, EMA and other foreign regulatory authority requirements on an ongoing basis. If we, or our CMOs, are unable to reliably produce products to specifications acceptable to the FDA, EMA or other foreign regulatory authorities, we may not obtain or maintain the approvals we need to commercialize such products. Even if we obtain regulatory approval for any of our product candidates, there is no assurance that either we or our CMOs will be able to manufacture the approved product to specifications acceptable to the FDA, EMA or other foreign regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product, or to meet potential future demand. Any of these challenges could delay completion of clinical trials, require bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidate, impair commercialization efforts, increase our cost of goods, and have an adverse effect on our business, financial condition, results of operations and growth prospects. Although we are working to develop commercially viable processes, our manufacturing capabilities could be affected by cost-overruns,

unexpected delays, equipment failures, labor shortages, natural disasters, power failures and numerous other factors that could prevent us from realizing the intended benefits of our manufacturing strategy and have a material adverse effect on our business. We may ultimately be unable to, among other things, develop a manufacturing process and distribution network that will, reduce the cost of goods for our product candidates to levels that will allow for an attractive return on investment if and when those product candidates are commercialized.

***Although we currently plan to retain all commercial rights to our product candidates, we may enter into strategic collaborations for the development, marketing and commercialization of all or some of our product candidates. If those collaborations are not successful, or if we are unable to establish any such collaborations, we may have to alter or delay our development and commercialization plans.***

As we further develop our lead product candidates, we may build a commercial infrastructure with the capability to directly market it to a variety of markets and territories. Although we currently plan to retain all commercial rights to our product candidates, we may enter into strategic collaborations for the development, marketing and commercialization of all or some of our product candidates. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. We would face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we do enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development, marketing and/or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development, marketing and/or commercialization of our product candidates or may elect not to continue or renew development, marketing or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, drugs that compete directly or indirectly with our drugs or product candidates;
- a collaborator with marketing and distribution rights to one or more drugs may not commit sufficient resources to the marketing and distribution of such drug or drugs;

- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- we may lose certain valuable rights under circumstances identified in any collaboration arrangement that we enter into, such as if we undergo a change of control;
- we may be restricted under then-existing collaboration agreements from entering into future agreements on certain terms with potential collaborators;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development, marketing and/or commercialization of the applicable product candidates;
- collaborators may learn about our discoveries, data, proprietary information, trade secrets, or compounds and use this knowledge to compete with us in the future; and
- the number and type of our collaborations could adversely affect our attractiveness to potential future collaborators or acquirers.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner, or at all.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all, if and when we seek to enter into collaborations. If we are unable to do so, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms, or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue from sales of drugs.

***Reimbursement decisions by third-party payors may have an adverse effect on pricing and market acceptance. If there is not sufficient reimbursement for our products, it is less likely that our products will be widely used.***

Even if our product candidates are approved for sale by the appropriate regulatory authorities, market acceptance and sales of these products will depend on reimbursement policies and may be affected by future healthcare reform measures. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will reimburse and establish payment levels and, in some cases, utilization management strategies, such as tiered formularies and prior authorization. We cannot be certain that reimbursement will be available for any products that we develop or that the reimbursement level will be adequate to allow us to operate profitably. Also, we cannot be certain that reimbursement policies will not reduce the demand for, or the price paid for, our products. If reimbursement is not available or is available on a limited basis, or

if the reimbursement amount is inadequate, we may not be able to successfully commercialize any of our approved products.

### **Risks Related to Government Regulation; Market Approval and Other Legal Compliance Matters**

*Even if we complete the necessary pre-clinical studies and clinical trials, the FDA regulatory approval process is lengthy, time-consuming, and inherently unpredictable, and we or any of our potential future collaborators may experience significant delays in the clinical development and regulatory approval, if any, for the commercialization of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or any of our potential future collaborators, will obtain marketing approval to commercialize a product candidate.*

The research, testing, manufacturing, labeling, approval, selling, import, export, marketing, and distribution of drug products, including biologics, are subject to extensive regulation by the FDA and other regulatory authorities in the United States. Even if we complete the necessary pre-clinical studies and clinical trials, we will not be permitted to market any biological drug product in the United States until we receive a Biologics License from the FDA. We plan to begin additional clinical trials with our lead product candidates in 2018 and 2019. We intend to conduct each of these clinical trials in the United States and Europe. We intend to discuss with the FDA and EMA submission of BLAs for respective approval of such product candidates as treatments for indications that currently lack FDA-approved treatments.

The FDA standard for regular approval of a BLA generally requires two well-controlled Phase 3 studies or one large and robust, well-controlled Phase 3 study in the patient population being studied that provides substantial evidence that a biologic is safe, pure and potent. Phase 3 clinical studies typically involve hundreds of patients, have significant costs and take years to complete. However, product candidates studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA may require a sponsor of a drug or biologic receiving accelerated approval to perform post-marketing studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint, and the drug or biologic may be subject to withdrawal procedures by the FDA that are more accelerated than those available for regular approvals. We believe our accelerated approval strategy is warranted given the currently limited alternative therapies for patients with pediatric relapsed or refractory, or R/R, from neuroblastoma, or NB, but the FDA may not agree. The FDA may ultimately require one or multiple Phase 3 clinical trials prior to approval.

We have not previously submitted a BLA to the FDA, or similar approval filings to comparable foreign authorities. A BLA must include extensive pre-clinical and clinical data and supporting information to establish that the product candidate is safe, pure, and potent for each desired indication. The BLA must also include significant information regarding the chemistry, manufacturing, and controls for the product, and the manufacturing facilities must complete a successful pre-license inspection. We expect the novel nature of our product candidates to create further challenges in obtaining regulatory approval from the FDA and other regulatory authorities. The FDA may also require a panel of experts, referred to as an Advisory Committee, to deliberate on the adequacy of the safety and efficacy data to support licensure. The opinion of the Advisory Committee, although not binding, may have a significant impact on our ability to obtain licensure of the product candidates

based on the completed clinical trials. Accordingly, the regulatory approval pathway for our product candidates may be uncertain, complex, expensive, and lengthy, and approval may not be obtained.

The process of obtaining marketing approvals, both in the United States and abroad, is a lengthy, expensive and uncertain process. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Securing marketing approval requires the submission of extensive pre-clinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. The FDA or other regulatory authorities have substantial discretion and may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

In addition, clinical trials can be delayed or terminated for a variety of reasons, including delays or failures related to:

- obtaining regulatory approval to begin a trial, if applicable;
- the availability of financial resources to begin and complete the planned trials;
- reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- obtaining approval at each clinical trial site by an IRB;
- recruiting suitable patients to participate in a trial in a timely manner;
- having patients complete a trial or return for post-treatment follow-up;
- clinical trial sites deviating from trial protocol, not complying with cGCPs, or dropping out of a trial;
- addressing any patient safety concerns that arise during the course of a trial;
- addressing any conflicts with new or existing laws or regulations;
- adding new clinical trial sites; or
- manufacturing qualified materials under cGMPs for use in clinical trials.

Patient enrollment is a significant factor in the timing of clinical trials and is affected by many factors. See the risk factor above "—The market opportunities for our product candidates may be limited to those patients who are ineligible for or have failed prior treatments and may be small. If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected." for additional information on risks related to patient enrollment. Further, a clinical trial may be suspended or terminated by us, the IRBs for the institutions in which such trials are being conducted, the Data Monitoring Committee for such trial, or the FDA or other regulatory authorities due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects for our product

candidates will be harmed, and our ability to generate potential future product revenue will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue.

Our third-party research institution collaborators may also experience similar difficulties in completing ongoing clinical trials and conducting future clinical trials of product candidates. Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Our product candidates could fail to receive marketing approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from pre-clinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of a BLA or other submission or to obtain marketing approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies;
- the FDA or comparable foreign regulatory authorities may fail to approve any companion diagnostics that may be required in connection with approval of our therapeutic product candidates; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process as well as the unpredictability of clinical trial results may result in our failing to obtain marketing approval to market any of our product candidates, which would significantly harm our business, results of operations and prospects.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted drug application may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional pre-clinical studies, clinical trials, toxicology or other *in vivo* or *in vitro* data to support the initiation of other studies and testing. In addition, varying interpretations of the data obtained from pre-clinical studies and clinical trials could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any collaborators we may have in the future, ultimately

obtain may be limited or subject to restrictions or post-approval commitments that render the approved drug not commercially viable.

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability or that of any collaborators we may have to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

***The European Medicines Agency, or the EMA, or comparable foreign regulatory authorities, may disagree with our regulatory plans, including our plans to seek accelerated approval, and we may fail to obtain regulatory approval of our product candidates, which would prevent our product candidates from being marketed abroad. Any approval we are granted for our product candidates in the United States would not assure approval of our product candidates in foreign jurisdictions.***

In order to market and sell our drugs in the European Union and many other jurisdictions, we, and any collaborators we may have in the future, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The marketing approval process outside of the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside of the United States, it is required that the drug be approved for reimbursement before the drug can be approved for sale in that country. We, and any collaborators we may have in the future, may not obtain approvals from regulatory authorities outside of the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside of the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

As part of its marketing authorization process, the EMA may grant marketing authorizations on the basis of less complete data than is normally required, when, for certain categories of medicinal products, doing so may meet unmet medical needs of patients and serve the interest of public health. In such cases, it is possible for the Committee for Medicinal Products for Human Use, or CHMP, to recommend the granting of a marketing authorization, subject to certain specific obligations to be reviewed annually, which is referred to as a conditional marketing authorization. This may apply to medicinal products for human use that fall under the jurisdiction of the EMA, including those that aim at the treatment, the prevention, or the medical diagnosis of seriously debilitating diseases or life-threatening diseases and those designated as orphan medicinal products.

A conditional marketing authorization may be granted when the CHMP finds that, although comprehensive clinical data referring to the safety and efficacy of the medicinal product have not been supplied, all the following requirements are met:

- the risk-benefit balance of the medicinal product is positive;
- it is likely that the applicant will be in a position to provide the comprehensive clinical data;
- unmet medical needs will be fulfilled; and
- the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required.

The granting of a conditional marketing authorization is restricted to situations in which only the clinical part of the application is not yet fully complete. Incomplete non-clinical or quality data may only be accepted if duly justified and only in the case of a product intended to be used in emergency situations in response to public-health threats.



Conditional marketing authorizations are valid for one year, on a renewable basis. The holder will be required to complete ongoing studies or to conduct new studies with a view to confirming that the benefit-risk balance is positive. In addition, specific obligations may be imposed in relation to the collection of pharmacovigilance data.

The granting of a conditional marketing authorization will allow medicines to reach patients with unmet medical needs earlier than might otherwise be the case and will ensure that additional data on a product are generated, submitted, assessed and acted upon. Although we may seek a conditional marketing authorization for one or more of our product candidates by the EMA, the EMA or CHMP may ultimately not agree that the requirements for such conditional marketing authorization have been satisfied.

Our clinical trial results may also not support approval, whether accelerated approval, conditional marketing authorizations, or regular approval. The results of pre-clinical and clinical studies may not be predictive of the results of later-stage clinical trials, and product candidates in later stages of clinical trials may fail to show the desired safety and efficacy despite having progressed through pre-clinical studies and initial clinical trials. In addition, our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- we may be unable to demonstrate that our product candidates' risk-benefit ratios for their proposed indications are acceptable;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that the clinical and other benefits of our product candidates outweigh their safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from pre-clinical studies or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the FDA or comparable foreign regulatory authorities to support the submission of a BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, our own manufacturing facilities, or a third-party manufacturer's facilities with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, the referendum could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the

European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

Failure to obtain regulatory approval to market any of our product candidates would significantly harm our business, results of operations, and prospects.

***We may seek BTM for naxitamab or one or more of our other product candidates. We may not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.***

In 2012, the FDA established BTM, which is intended to expedite the development and review of products that treat serious or life-threatening diseases when "preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development." The designation of a product candidate as a breakthrough therapy provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate and ensure collection of appropriate data needed to support approval; more frequent written correspondence from FDA about such things as the design of the proposed clinical trials and use of biomarkers; intensive guidance on an efficient drug development program, beginning as early as Phase 1; organizational commitment involving senior managers; and eligibility for rolling review and priority review.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of 10 months. We may request priority review for our product candidates. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all. BTM does not change the standards for product approval.

In June 2017, BTM for <sup>131</sup>I-omburtamab was received for the treatment of pediatric patients with R/R NB who have CNS or LM. We may seek BTM for some or all of our other product candidates, but we may never receive such BTM, or, if received, the development of our product candidates may not be expedited or benefited by such designation.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive BTM, the receipt of such designation for a product candidate may not result in a faster development or regulatory review or approval process compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

***Our product candidates may not be able to obtain ODD or RPDD or obtain or maintain orphan drug exclusivity. We will not be eligible to receive PRVs in the event that our product candidates are not approved before October 1, 2022.***

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In August 2016, the FDA granted ODD to omburtamab for the treatment of NB. In April 2017, the EMA granted ODD to omburtamab for the treatment of CNS/LM from NB.

In the United States, ODD entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has ODD subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity in the United States provides that the FDA may not approve any other applications, including a full BLA, to market the same drug for the same indication for seven years, except in limited circumstances. The applicable exclusivity period is 10 years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for ODD or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

In 2012, the United States Congress effectuated a Rare Pediatric Disease Priority Review Voucher Program, or PRV Program, to incentivize pharmaceutical sponsors to develop drugs for rare pediatric diseases. A sponsor who obtains approval of a New Drug Application or BLA for a rare pediatric disease may be eligible for a Priority Review Voucher, or PRV, under this program, which may be redeemed by the owner of such PRV to obtain priority review for a marketing application. A PRV is fully transferrable and can be sold to any sponsor, who in turn can redeem the PRV for priority review of a marketing application in six months, compared to the standard timeframe of approximately 10 months. The terms of our license agreements with MSK provide that MSK is entitled to receive 40-50% of any income generated from the sale of any PRV or the sale of any other comparable incentives provided by any non-U.S. jurisdiction that we may receive. In December 2016, the 21st Century Cures Act, or the Cures Act, became effective, which, among other initiatives, reauthorized the PRV Program until 2020. Under the Cures Act, a drug that receives RPDD before October 1, 2020, will continue to be eligible for a PRV if the drug is approved before October 1, 2022.

Even if our other product candidates obtain ODD or RPDD in the future, they may not be able to obtain or maintain orphan drug exclusivity, priority review or expedited regulatory approval for that product candidate. We may not be the first to obtain marketing approval of any product candidate that has obtained ODD for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties may be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care or the manufacturer of the product with orphan exclusivity is unable to maintain sufficient product quantity. ODD neither shortens

the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

***Even if we, or any collaborators we may have in the future, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our drugs could require substantial expenditure of resources and may limit how we, or they, manufacture and market our drugs, which could materially impair our ability to generate revenue.***

Once marketing approval has been granted, an approved drug and its manufacturer and marketer are subject to ongoing review and extensive regulation. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. We, and any collaborators we may have in the future, must also comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the drug's approved labeling. Thus, we, and any collaborators we may have in the future, may not be able to promote any drugs we develop for indications or uses for which they are not approved.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a drug. For example, the approval may be subject to limitations on the indicated uses for which the drug may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and Mitigation Strategy, which could include requirements for a restricted distribution system. Manufacturers of approved drugs and those manufacturers' facilities are also required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, our future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or our potential future collaborators, receive marketing approval for one or more of our product candidates, we, and our potential future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and our future potential collaborators, are not able to comply with post-approval regulatory requirements, we, and our potential future collaborators, could have the marketing approvals for our drugs withdrawn by regulatory authorities and our, or our potential future collaborators', ability to market any future drugs could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

***The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates, which would impact our ability to generate revenue.***

In December 2016, the Cures Act was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and spur innovation, but its ultimate implementation is unclear. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any

marketing approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump administration may impact our business and industry. Namely, the Trump administration has taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. Notably, on January 30, 2017, President Trump issued an Executive Order, applicable to all executive agencies, including the FDA, which requires that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This Executive Order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the Executive Order requires agencies to identify regulations to offset any incremental cost of a new regulation and approximate the total costs or savings associated with each new regulation or repealed regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within OMB on February 2, 2017, the administration indicates that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. In addition, on February 24, 2017, President Trump issued an Executive Order directing each affected agency to designate an agency official as a "Regulatory Reform Officer" and establish a "Regulatory Reform Task Force" to implement the two-for-one provisions and other previously issued Executive Orders relating to the review of federal regulations; however, it is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

***Any of our product candidates for which we, or our potential future collaborators, obtain marketing approval in the future will be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our drugs following approval.***

Any of our product candidates for which we, or our potential future collaborators, obtain marketing approval in the future, will be subject to continual review by the FDA and other regulatory authorities.

The FDA and other agencies, including the Department of Justice, or the DOJ, closely regulate and monitor the post-approval marketing and promotion of drugs to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or our potential future collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the Food, Drug and Cosmetic Act of 1938, or FDCA, and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our drugs or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- litigation involving patients taking our drug;
- restrictions on such drugs, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a drug;
- restrictions on drug distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the drugs from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of drugs;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- restrictions on coverage by third-party payors;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of drugs;
- drug seizure; or
- injunctions or the imposition of civil or criminal penalties.

***Current and future legislation, and a change in existing government regulations and policies, may increase the difficulty and cost for us and our potential future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.***

In the United States and some foreign jurisdictions, there have been and continue to be a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of our potential future collaborators, to profitably sell any drugs for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and additional downward pressure on the price that we, or our potential future collaborators, may receive for any approved drugs.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, changed the way Medicare covers and pays for pharmaceutical products and could decrease the coverage and price that we, or our potential future collaborators, may receive for any approved drugs. While the MMA only addresses drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, then-President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the

Affordable Care Act, or ACA, which substantially changes the way healthcare is financed by both governmental and private insurers. The provisions of the Affordable Care Act of potential importance to our product candidates are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum Medicaid rebates a manufacturer must pay under the Medicaid Drug Rebate Program and extension of the rebate program to individuals enrolled in Medicaid managed care organizations;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for non-compliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs and therapeutic biologics to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs and therapeutic biologics to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- new requirements to report certain financial arrangements with physicians and certain others, including reporting "transfers of value" made or distributed to prescribers and other healthcare providers and reporting investment interests held by physicians and their immediate family members;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- a new Independent Payment Advisory Board, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes included the Budget Control Act of 2011, which, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that started in 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2024 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used. More recently, President Trump has suggested that he plans to seek repeal of all or portions of the ACA, and he has indicated that he wants Congress to replace the ACA with new legislation. We cannot predict whether these challenges will continue or other proposals will be made or adopted, or what impact these efforts may have on us. Further, there has been heightened governmental scrutiny over the

manner in which manufacturers set prices for their marketed products, which has resulted in several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the price of drugs under Medicare and reform government program reimbursement methodologies for drug products. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

Some of the provisions of the ACA have yet to be implemented, and there have been legal and political challenges to certain aspects of the ACA. Since January 2017, President Trump has signed two executive orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the ACA. Moreover, the Tax Cuts and Jobs Act of 2017, or the Tax Reform Bill, was enacted on December 22, 2017, and includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Congress may consider other legislation to repeal or replace additional elements of the ACA. We continue to evaluate the effect that the ACA, the repeal of the individual mandate, and any additional possible repeal and replacement efforts may have on our business but expect that the ACA, as currently enacted or as it may be amended in the future, and other healthcare reform measures that may be adopted in the future could have a material adverse effect on our industry generally and on our ability to maintain or increase sales of our existing products that we successfully commercialize or to successfully commercialize our product candidates, if approved. In addition to the ACA, there will continue to be proposals by legislators at both the federal and state levels, regulators and third party payors to keep healthcare costs down while expanding individual healthcare benefits.

Additional changes that may affect our business include the expansion of new programs such as Medicare payment for performance initiatives for physicians under the Medicare Access and CHIP Reauthorization Act of 2015, which will be fully implemented in 2019. At this time, it is unclear how the introduction of the Medicare quality payment program will impact overall physician reimbursement. The costs of prescription pharmaceuticals in the United States has also been the subject of considerable discussion in the United States, and members of Congress and the Administration have stated that they will address such costs through new legislative and administrative measures. This focus has resulted in several Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the



amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our drug candidates or additional pricing pressures.

The cost of prescription pharmaceuticals in the United States has also been the subject of considerable discussion in the United States, and members of Congress and the Administration have stated that they will address such costs through new legislative and administrative measures. The pricing of prescription pharmaceuticals is also subject to governmental control outside the United States. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidates to that of other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our ability to generate revenues and become profitable could be impaired.

Legislative and regulatory proposals have also been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and any future collaborators to more stringent drug labeling and post-marketing testing and other requirements.

***Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our lead product candidates, if approved, or any of our other product candidates that may be approved in the future, which would adversely affect our revenue and results of operations.***

We expect that coverage and reimbursement of pharmaceutical products may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. In particular, drug pricing by pharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the U.S. and abroad. Government and private third-party payors have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the U.S. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payors, which may render our product candidates, if approved, not commercially viable or may adversely affect our anticipated future revenues and gross margins.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of pharmaceutical drugs generally could restrict the amount that we are able to charge for our future products, which would adversely affect our anticipated revenue and results of operations.

***Our relationships with healthcare providers, physicians and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties, including criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Our relationships with healthcare providers, physicians and third-party payors will subject us to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in which we conduct our business. Our future arrangements with healthcare providers, physicians and third-party payors and patients may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- *Anti-Kickback Statute*—the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- *False Claims Act*—the federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at \$5,500 to \$11,000 per false claim;
- *HIPAA*—the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and its implementing regulations, also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouse as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms and technical safeguards, with respect to safeguarding the privacy, security and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;
- *HIPAA Privacy Provisions*—as amended by HITECH and its implementing regulations, HIPAA also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouse as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms and technical safeguards, with respect to safeguarding the privacy, security and transmission of individually identifiable health information, and require

notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;

- *Transparency Requirements*—the federal legislation commonly referred to as the Physician Payments Sunshine Act, enacted as part of the Affordable Care Act, and its implementing regulations, which requires certain manufacturers of drugs, devices, therapeutic biologics and medical supplies reimbursable under Medicare, Medicaid, and Children's Health Insurance Programs to report annually to the Department of Health and Human Services information related to certain payments and other transfers of value, including consulting fees, travel reimbursements, research grants, and other payments or gifts with values over \$10 made to physicians, other healthcare providers and teaching hospitals, as well as ownership and investment interests held by physicians and other healthcare providers and their immediate family members;
- *FDCA*—the FDCA, which prohibits, among other things, the adulteration or misbranding of drugs, biologics and medical devices; and
- *Analogous State and Foreign Laws*—analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and pricing information. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, it may be costly to us in terms of money, time and resources, and they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

***Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our product candidates profitably.***

Successful sales of our product candidates, if approved, depend on the availability of adequate coverage and reimbursement from third-party payors. In addition, because our product candidates represent relatively new approaches to the treatment of cancer, we cannot accurately estimate the potential revenue from our product candidates.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors are critical to new product acceptance.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Coverage and reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of our products, if approved. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates. Because our product candidates have a higher cost of goods than conventional therapies, and may require long-term follow up evaluations, the risk that coverage and reimbursement rates may be inadequate for us to achieve profitability may be greater.

We intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of biologics is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of adequate coverage and reimbursement from third-party payors for our product candidates and may be affected by existing and future health care reform measures.

***Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and negligent conduct that fails to: comply with the laws of the FDA and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies; comply with manufacturing standards we have established; comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws; or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin

commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase significantly. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous, radioactive and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of hazardous and flammable materials, including chemicals and biological materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or commercialization efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

***Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.***

We currently have operations in the United States and Denmark and we maintain relationships with CMOs in other parts of Europe as well as in the United States for the manufacture of our product candidates. If we further expand our operations outside of the United States, we must comply with numerous laws and regulations in each new jurisdiction in which we plan to operate. The creation and implementation of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required. We cannot

assure you that our compliance policies and procedures are or will be sufficient or that our directors, officers, employees, representatives, consultants and agents have not engaged and will not engage in conduct for which we may be held responsible, nor can we assure you that our business partners have not engaged and will not engage in conduct that could materially affect their ability to perform their contractual obligations to us or even result in our being held liable for such conduct.

The FCPA prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the DOJ. The Securities and Exchange Commission, or SEC, is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain drugs and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA or other export control, anti-corruption, anti-money laundering and anti-terrorism laws or regulations can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

***The impact of the Tax Reform Bill could have a negative effect on us or our stockholders.***

On December 20, 2017, Congress passed the Tax Reform Bill and on December 22, 2017, President Trump signed the Tax Reform Bill into law. The Tax Reform Bill makes significant changes to the U.S. federal income tax rules applicable to both individuals and entities, including corporations. There is significant uncertainty as to the impact of the Tax Reform Bill on us, including, but not limited to, our ability to utilize our net operating loss carry forwards, and on any investment in our common stock. For losses arising in tax years beginning after December 31, 2017, the amount of net operating losses that we can use to offset taxable income is limited to 80% of our taxable income. You should consult with your tax advisor with respect to the status of U.S. federal tax reform and its potential effect on your investment in our common stock.

## Risks Related to Our Intellectual Property

***Our success depends in part on our ability to protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.***

Our commercial success will depend in large part on obtaining and maintaining patent, trademark and trade secret protection of our product candidates, products and related proprietary technologies, their respective components, formulations, methods used to manufacture them and methods of treatment, as well as successfully defending these patents against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell or importing our product candidates or products and related proprietary technologies is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities.

The patenting process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not be able to pursue or obtain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Our pending and future patent applications may not result in issued patents that protect our product candidates, products or related technologies, in whole or in part. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing product candidates or products and related technologies.

***We currently depend on proprietary technology licensed from MSK and may depend on other third party licensors in the future. If we lose our existing licenses or are unable to acquire or license additional proprietary rights from MSK or other third parties, we may not be able to continue developing our products.***

We currently in-license certain intellectual property from MSK. In the future we may in-license intellectual property from other licensors. We rely on certain of these licensors to file and prosecute patent applications and maintain patents and otherwise protect the intellectual property we license from them. We have limited control over these activities or any other intellectual property that may be related to our in-licensed intellectual property. For example, we cannot be certain that such activities by these licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. We have limited control over the manner in which our licensors initiate an infringement proceeding against a third-party infringer of the intellectual property rights, or defend certain of the intellectual property that is licensed to us. It is possible that the licensors' infringement proceeding or defense activities may be less vigorous than had we conducted them ourselves.

The growth of our business may depend in part on our ability to acquire or in-license additional proprietary rights. For example, our programs may involve additional product candidates that may require the use of additional proprietary rights held by third parties. Our product candidates or products may also require specific formulations to work effectively and efficiently. These formulations may be covered by intellectual property rights held by others. We may develop products containing our compounds and pre-existing pharmaceutical compounds. These pharmaceutical compounds may be covered by intellectual property rights held by others. We may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates. These diagnostic test or tests may be covered by intellectual property rights held by others. We may be unable to acquire or in-license any relevant third-party intellectual property rights that we identify as necessary or important to our business operations. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all, which would harm our business. We may need to cease use of the compositions or methods covered by such third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on such intellectual

property rights which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license under such intellectual property rights, any such license may be non-exclusive, which may allow our competitors to access the same technologies licensed to us.

Additionally, we sometimes collaborate with academic and other institutions, such as MSK, to accelerate our pre-clinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of such program and our business and financial condition could suffer.

The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates, products and related proprietary technologies. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. There can be no assurance that we will be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

We are a party to license agreements with MSK and others, pursuant to which we in-license key patent and patent applications for our product candidates, products and related proprietary technologies. These existing licenses impose various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations or otherwise materially breach a license agreement, our licensors may have the right to terminate the license, in which event we would not be able to develop or market the products covered by such licensed intellectual property. In addition, any claims asserted against us by our licensors may be costly and time-consuming, divert the attention of key personnel from business operations or otherwise have a material adverse effect on our business.

***Uncertainty as to the issuance, scope, validity, enforceability and value of patents, and the potential for future changes in patent and other intellectual property protections, may result in inadequate protection of our as well as in-licensed intellectual property or may result in alleged or actual infringement of the intellectual property rights of third parties.***

The patent position of pharmaceutical and biotechnology companies generally is highly uncertain and involves complex legal and factual questions for which many legal principles remain unresolved. In recent years patent rights have been the subject of significant litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights and in-licensed patent rights are highly uncertain. Our pending and future patent applications and in-licensed patent applications may not result in patents being issued in the United States or in other jurisdictions which protect our product candidates, products or related technologies or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our and in-licensed patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months



after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. In addition, the U.S. Patent and Trademark Office, or USPTO, might require that the term of a patent issuing from a pending patent application be disclaimed and limited to the term of another patent that is commonly owned or names a common inventor. As a result, the issuance, scope, validity, enforceability and commercial value of our as well as in-licensed patent rights are highly uncertain.

Recent or future patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our and in-licensed patent applications and the enforcement or defense of the issued patents. In March 2013, under the Leahy-Smith America Invents Act, or America Invents Act, the United States moved from a "first to invent" to a "first-to-file" system. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. The America Invents Act includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, redefine prior art and establish a new post-grant review system. The effects of these changes are currently unclear as the USPTO only recently developed new regulations and procedures in connection with the America Invents Act and many of the substantive changes to patent law, including the "first-to-file" provisions, only became effective in March 2013. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition. We may become involved in opposition, interference, derivation, *inter partes* review or other proceedings challenging our patent rights or the patent rights of others, and the outcome of any proceedings are highly uncertain. An adverse determination in any such proceeding could reduce the scope of, or invalidate, our and in-licensed patent rights, allow third parties to commercialize our products, product candidates and related technologies and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

***Intellectual property rights do not necessarily address all potential threats.***

Even if our or in-licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. The issuance of a patent is not conclusive as to its scope, validity or enforceability, and our owned and in-licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the patent claims of our owned or in-licensed patents being narrowed, invalidated or held unenforceable, which could limit our ability to stop or prevent us from stopping others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our product candidates, products and technology. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our and in-licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours or otherwise provide us with a competitive advantage.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds that are similar to the pharmaceutical compounds used in our product candidates or products but that are not covered by the claims of our patents;
- the APIs in our current product candidates may eventually become commercially available in generic drug products, and no patent protection may be available with regard to formulation or method of use;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government in regards to any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss of patent rights;
- we or our licensors, as the case may be, might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our product candidates or products and proprietary technologies;
- it is possible that our or in-licensed pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' patents, as the case may be, or parts of our or their patents;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates, products or technologies similar to ours;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates or products;
- our owned or in-licensed issued patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes which design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- we have engaged in scientific relationships in the past, such as with MSK, and expect to continue to do so with MSK and/or other third parties in the future. Such third parties may develop adjacent or competing products to ours that are outside the scope of our licensed patents and/or the respective research collaboration/agreement with such third party;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or

- the patents of others may have an adverse effect on our business.

In addition, during the course of business we have decided not to pursue certain products or processes and we may do so again in the future. If it is later determined that our activities or product candidates infringe this intellectual property we may be liable for damages, enhanced damages or subjected to an injunction, any of which could have a material adverse effect on our business.

We also may rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect, and we have limited control over the protection of trade secrets used by our licensors, collaborators and suppliers. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our information to competitors or use such information to compete with us. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. If our confidential or proprietary information is divulged to or acquired by third parties, including our competitors, our competitive position in the marketplace will be harmed and this would have a material adverse effect on our business.

If any of our owned or in-licensed patents are found to be invalid or unenforceable, or if we are otherwise unable to adequately protect our rights, it could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates. Likewise, our current owned and in-licensed patents covering our proprietary technologies and our product candidates are expected to expire on various dates from 2021 through 2031, without taking into account any possible patent term adjustments or extensions. Our earliest in-licensed patents were only filed in the United States and may expire before, or soon after, our first product achieves marketing approval in the United States. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, results of operations, financial condition and prospects. We own or in-license pending patent applications covering our proprietary technologies or our product candidates that if issued as patents are expected to expire from 2021 through 2035 (2038 assuming the future filing of a priority claiming Patent Cooperation Treaty application), without taking into account any possible patent term adjustments or extensions. However, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of these patent applications.

***We may incur substantial costs as a result of litigation or other proceedings relating to patents, and we may be unable to protect our rights to our product candidates, products and technologies.***

If we or our licensors choose to go to court to stop a third party from using the inventions claimed in our owned or in-licensed patents, that third party may ask the court to rule that the patents are invalid and/or should not be enforced against that third party. These lawsuits are expensive and would consume time and other resources even if we or they, as the case may be, were successful in stopping the infringement of these patents. In addition, there is a risk that the court will decide that these patents are not valid and that we or they, as the case may be, do not have the right to stop others from using the inventions.

There is also the risk that, even if the validity of these patents is upheld, the court will refuse to stop the third party on the ground that such third party's activities do not infringe our owned or in-licensed patents. In addition, the U.S. Supreme Court has recently changed some legal principles that affect patent applications, granted patents and assessment of the eligibility or validity of these patents. As a consequence, issued patents may be found to contain invalid claims according to the newly revised eligibility and validity standards. Some of our owned or in-licensed patents may be subject to challenge and subsequent invalidation or significant narrowing of claim scope in proceedings

before the USPTO, or during litigation, under the revised criteria which could also make it more difficult to obtain patents.

We, or our licensors, may not be able to detect infringement against our owned or in-licensed patents, as the case may be, which may be especially difficult for manufacturing processes or formulation patents. Even if we or our licensors detect infringement by a third party of our owned or in-licensed patents, we or our licensors, as the case may be, may choose not to pursue litigation against or settlement with the third party. If we, or our licensors, later sue such third party for patent infringement, the third party may have certain legal defenses available to it, which otherwise would not be available except for the delay between when the infringement was first detected and when the suit was brought. Such legal defenses may make it impossible for us or our licensors to enforce our owned or in-licensed patents, as the case may be, against such third party.

If another party questions the patentability of any of our claims in our owned or in-licensed U.S. patents, the third party can request that the USPTO review the patent claims such as in an *inter partes* review, *ex parte* re-exam or post-grant review proceedings. These proceedings are expensive and may result in a loss of scope of some claims or a loss of the entire patent. In addition to potential USPTO review proceedings, we may become a party to patent opposition proceedings in the European Patent Office, or EPO, or similar proceedings in other foreign patent offices, where either our owned or in-licensed foreign patents are challenged. The costs of these opposition or similar proceedings could be substantial, and may result in a loss of scope of some claims or a loss of the entire patent. An unfavorable result at the USPTO, EPO or other patent office may result in the loss of our right to exclude others from practicing one or more of our inventions in the relevant country or jurisdiction, which could have a material adverse effect on our business.

***We may incur substantial costs as a result of litigation or other proceedings relating to intellectual property rights other than patents, and we may be unable to protect our rights to our product candidates, products and technologies.***

We may rely on trade secrets and confidentiality or nondisclosure agreements to protect our proprietary technology and know-how, especially where we do not believe patent protection is appropriate or obtainable. Where we enter into agreements imposing confidentiality or nondisclosure obligations upon employees or third parties to protect our proprietary technology and know-how, these confidentiality obligations may be breached or may not provide meaningful protection for our trade secrets or proprietary technology and know-how. Furthermore, despite the existence of such confidentiality and nondisclosure agreements, or other contractual restrictions, we may not be able to prevent the unauthorized disclosure or use of our confidential proprietary information or trade secrets by consultants, vendors, former employees or current employees. In addition, adequate remedies may not be available in the event of an unauthorized access, use, or disclosure of our trade secrets or know-how.

Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States may be less willing to protect trade secrets effectively or to the same extent as the laws of the United States. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. These lawsuits may consume our time and other resources even if we are successful.

Third parties may obtain knowledge of our trade secrets through independent development or other access by legal means. The occurrence of such events could limit or preclude our ability to produce or sell our products in a competitive manner or otherwise have a material adverse effect on our business.

***If we are sued for infringing patents or other intellectual property rights of third parties, it will be costly and time consuming, and an unfavorable outcome in that litigation may have a material adverse effect on our business.***

Our commercial success depends upon our ability to develop, manufacture, market and sell our product candidates or products and use our proprietary technologies without infringing the proprietary rights of third parties. U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields relating to our product candidates or products. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that others may assert that our product candidates or products infringe others' patent rights. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our fields, there may be a risk that third parties may allege they have patent rights encompassing our product candidates or products, technologies or methods.

In addition, because some patent applications in the United States may be maintained in secrecy until the patents are issued, patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our owned and in-licensed issued patents or our pending applications, or that we or, if applicable, a licensor were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Any such patent application may have priority over our owned and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to those owned by or in-licensed to us, we or, in the case of in-licensed technology, the licensor may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. If we or one of our licensors is a party to an interference proceeding involving a U.S. patent application on inventions owned by or in-licensed to us, we may incur substantial costs, divert management's time and expend other resources, even if we are successful.

There is a substantial amount of litigation involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries generally. We may be subject to, or threatened with litigation by third parties having patent or other intellectual property rights alleging that our product candidates or products and/or proprietary technologies infringe, misappropriate or violate their intellectual property rights.

If a third party claims that we infringe its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims which, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages and the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates or products, or from using our proprietary technologies, unless the third party licenses its product rights to us, which it is not required to do;

- if a license is available from a third party, it may not be offered on reasonable terms and may require that we pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our products; and
- redesigning our product candidates or products or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

We may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in an *ex-parte* re-exam, *inter partes* review or post-grant review proceedings. These proceedings are expensive and may consume our time or other resources. We may choose to challenge a third party's patent in patent opposition proceedings in the EPO, or other foreign patent office. The costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates or products or proprietary technologies.

***We may not be able to protect our intellectual property rights with patents throughout the world.***

Filing, prosecuting and defending patents on all of our product candidates or products throughout the world would be prohibitively expensive. Competitors may use our technology in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as in the United States. These products may compete with our product candidates or products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our proprietary rights generally. The initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

***Obtaining and maintaining our patent protection depends upon compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent prosecution process and following the issuance of a patent. Our failure to comply with such requirements could result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case if our patent were in force, which would have a material adverse effect on our business.

***We may be subject to claims that our licensors, employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their former employers or their clients.***

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although no claims against us are currently pending, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

***We have not yet registered our trademarks in the United States. Failure to secure such registrations could adversely affect our business.***

We have not yet registered our trademarks in the United States. If we do not successfully register our trademarks, we may encounter difficulty in enforcing, or be unable to enforce, our trademark rights against third parties, which could adversely affect our business and our ability to effectively compete in the marketplace. We have also not yet registered trademarks for any of our product candidates in any jurisdiction. When we file registration applications for trademarks relating to our product candidates, those applications may be rejected, and registered trademarks may not be obtained, maintained or enforced. During trademark registration proceedings in the United States and foreign jurisdictions, we may receive rejections. We are given an opportunity to respond to those rejections, but we may not be able to overcome such rejections. In addition, in the United States Patent and Trademark Office and in comparable agencies in many foreign jurisdictions, third parties may oppose pending trademark registration applications or seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademark registrations may not survive such proceedings.

In addition, any proprietary name we propose to use with any of our product candidate in the United States must be approved by the FDA, regardless of whether we have registered, or applied to register, the proposed proprietary name as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable proprietary product name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

***If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest, which may have a material adverse effect on our business.***

We rely on our trademarks, trade names, service marks, domain names and logos, as appropriate, to market our brands and to build and maintain brand recognition. We rely on trademark protections to protect our business and our products and services. We generally seek to register and continue to register and renew, or secure by contract where appropriate, trademarks, trade names and service marks as they are developed and used, and reserve, register and renew domain names as appropriate. Our registered trademarks, if any, or unregistered trademarks, trade names or service marks may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. Effective trademark protection may not be available or may not be sought in every country in which our products are made available and contractual disputes may affect the use of marks governed by private contract. Similarly, not every variation of a domain name may be available or be registered, even if available. We may not be able to protect our rights to these trademarks, trade names, service marks and domain names, which we need to build brand name recognition in our markets of interest. And while we seek to protect the trademarks we use in the United States and in other countries, we may be unsuccessful in obtaining registrations and/or otherwise protecting these trademarks. If that were to happen, we may be prevented from using our names, brands and trademarks unless we enter into appropriate royalty, license or coexistence agreements. Over the long term, if we are unable to establish name recognition based on our trademarks, trade names, service marks and domain names, then we may not be able to compete effectively, resulting in a material adverse effect on our business.

#### **Risks Related to Employee Matters and Managing Growth**

***We have a limited number of employees and depend heavily on our executive officers and consultants. Our future success depends on our ability to retain our senior management and other key executives and to attract, retain and motivate qualified personnel. The loss of their services could materially harm our business.***

We are highly dependent on Thomas Gad, our Founder, Chairman, President and Head of Business Development; Dr. Claus Juan Møller San Pedro, M.D., Ph.D., our Chief Executive Officer; Bo Kruse, our Executive Vice President, Chief Financial Officer, Secretary and Treasurer; Joris Wiel Jan Wilms, our Senior Vice President and Chief Operating Officer; Dr. Torben Lund-Hansen, Ph.D., our Senior Vice President and Head of Technical Operations; and Dr. Steen Lisby, M.D., DMSc, our Senior Vice President and Chief Medical Officer, as well as the other principal members of our management and scientific teams. Our agreements with our executive officers do not prevent them from terminating their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. We intend to conduct our operations in the New York City metropolitan area, in a region that is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel is intense and the turnover rate can be high, which may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. We expect that we will need to recruit talent from outside of our region, and doing so may be costly and difficult.

To induce valuable employees to join and remain at our company, in addition to salary and cash incentives, we have provided, and intend to continue to provide, stock option and/or restricted stock grants that vest over time. The value to employees of these equity grants that vest over time may be significantly affected by movements in the fair market value of our capital stock that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.



Although we have employment agreements with our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice.

In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

***We expect to expand our development and regulatory capabilities and our sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical operations, regulatory affairs and, potentially, sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

#### **Risks Related to Our Common Stock and This Offering**

***After this offering, our executive officers, directors and principal stockholders will maintain ownership of a significant percentage of our stock and will be able to exercise significant influence over matters subject to stockholder approval.***

Upon the closing of this offering, our executive officers and directors and our existing stockholders, which own more than 5% of our outstanding common stock before this offering, will, in the aggregate, beneficially own shares representing approximately % of our common stock, not including any shares purchased by these stockholders in this offering. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

***Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.***

Provisions in our certificate of incorporation and our bylaws that will become effective upon the closing of this offering may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our

stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, or DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

***If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.***

The initial public offering price of shares of our common stock is substantially higher than the as adjusted net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this initial public offering, you will pay a price per share of common stock that substantially exceeds our as adjusted net tangible book value per share of common stock after this initial public offering. To the extent shares of our common stock are issued under outstanding options, you will incur further dilution. Based on the initial public offering price of \$ \_\_\_\_\_ per share of common stock, which is the midpoint of the price range set forth on the cover of this prospectus, you will experience immediate dilution of \$ \_\_\_\_\_ per share of common stock, representing the difference between our as adjusted net tangible book value per share of common stock after giving effect to this offering and the assumed initial public offering price per share of common stock. In addition, purchasers of shares of our common stock in this offering will have contributed approximately \_\_\_\_\_ % of the aggregate price paid by all purchasers of shares of our common stock but will own only approximately \_\_\_\_\_ % of our common stock outstanding after this offering.

***An active trading market for our common stock may not develop and, as a result, it may be difficult for you to sell your shares of our common stock.***

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters.

Although we plan to list our common stock on the Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop or is not sustained, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for the shares, or at all.

***If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.***

The trading market for our common stock will rely in part on the research and reports that industry or financial analysts publish about us or our business. We do not currently have and may never obtain research coverage by industry or financial analysts. If no or few analysts commence coverage of us, the trading price of our stock would likely decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

***The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.***

Our stock price is likely to be volatile. The stock market in general and the market for pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- the timing and results of clinical trials of any of our product candidates;
- regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- the success of existing or new competitive products or technologies;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- establishment or termination of collaborations for our product candidates or development programs;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results or development timelines;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;

- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

***We could be subject to securities class action litigation.***

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and our resources, which could harm our business.

***We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.***

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

***We are an "emerging growth company" and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.***

We are an "emerging growth company" as defined in the JOBS Act. We may remain an emerging growth company for up to five years, or until such earlier time as we have more than \$1.07 billion in annual revenue, the market value of our stock held by non-affiliates is more than \$700 million or we issue more than \$1 billion of non-convertible debt over a three-year period. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, being permitted to present only two years of audited financial statements and a correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this prospectus, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. In this prospectus, we have not included all of the executive compensation related information that would be required if we were not an emerging growth company. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

***We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.***

As a public company, and particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. There is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

***Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.***

Utilization of net operating loss carry forwards depends on many factors, including our future income, which cannot be assured, and the impact of the Tax Reform Bill. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change" (generally defined as a greater than 50-percentage-point cumulative change (by value) in the equity ownership of certain stockholders over a rolling three-year period), the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change taxable income or taxes may be limited. As a result of our current and planned fund raising activities, including this offering, we may experience such an "ownership change." We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which changes are outside our control. As a result, our ability to use any pre-change net operating loss carryforwards and other pre-change tax attributes to offset post-change

taxable income or taxes may be subject to limitation and may harm our future operating results by effectively increasing our future tax obligations.

***Because we do not anticipate paying any cash dividends on our capital stock for the foreseeable future, capital appreciation, if any, of our common stock will be your sole source of gain.***

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

***A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have outstanding \_\_\_\_\_ shares of common stock based on the number of shares outstanding as of \_\_\_\_\_, 2018. Of these shares of our common stock, \_\_\_\_\_ shares to be sold in this offering, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable, without restriction, in the public market immediately following this offering, unless purchased by our affiliates. All of the remaining shares are currently restricted as a result of securities laws or lock-up agreements but will be able to be sold after the offering as described in the "Shares Eligible for Future Sale" section of this prospectus, including with the prior written consent of Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC during the term of the lock-up agreements. Moreover, after this offering, holders of an aggregate of shares of our common stock will have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriting" section of this prospectus.

***Our certificate of incorporation that will become effective upon the closing of this offering designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against us and our directors, officers and employees.***

Our certificate of incorporation that will become effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the DGCL or our certificate of incorporation or bylaws, or any action asserting a claim against us governed by the internal affairs doctrine. This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees.

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among other things, statements about:

- the implementation of our business model and our plans to develop and commercialize our lead product candidates and other product candidates, including the potential benefits thereof;
- our ongoing and future clinical trials for our lead product candidates, whether conducted by us or by any of our collaborators, including the timing of initiation of these trials and of the anticipated results;
- our pre-clinical studies and future clinical trials for our other product candidates and our research and development programs, whether conducted by us or by any of our collaborators, including the timing of initiation of these trials and of the anticipated results;
- the timing of and our ability to obtain and maintain regulatory and marketing approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- the pricing and reimbursement of our product candidates, if approved;
- our ability to retain the continued service of our key employees and to identify, hire and retain additional qualified employees;
- remediation of material weaknesses in our internal control over financial reporting;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy and the scope of protection we are able to establish and maintain for the intellectual property rights covering our product candidates and technology;
- our ability to identify and develop additional product candidates and technologies with significant commercial potential;
- our plans and ability to enter into collaborations or strategic partnerships for the development and commercialization of our product candidates and future operations;
- the potential benefits of any future collaboration or strategic partnerships;
- our expectations related to the use of proceeds from this offering and our existing cash and cash equivalents;
- our financial performance, including our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- developments relating to our competitors and our industry;

- the impact of government laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the "Risk Factors" section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make or enter into.



## USE OF PROCEEDS

We estimate that the net proceeds to us from our issuance and sale of \_\_\_\_\_ shares of our common stock in this offering will be approximately \$ \_\_\_\_\_ million, assuming an initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise in full their option to purchase additional shares, we estimate that the net proceeds from this offering will be approximately \$ \_\_\_\_\_ million.

A \$1.00 increase or decrease in the assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease our net proceeds from this offering by approximately \$ \_\_\_\_\_ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. An increase or decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase or decrease our net proceeds from this offering by approximately \$ \_\_\_\_\_ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

As of December 31, 2017, we had cash and cash equivalents of \$ \_\_\_\_\_ million. We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately \$ \_\_\_\_\_ million to fund our ongoing pivotal stage development through regulatory submission, and other clinical development and expansion into new indications of one of our lead product candidates, naxitamab;
- approximately \$ \_\_\_\_\_ million to fund our ongoing pivotal stage development through regulatory submission, and other clinical development and expansion into new indications of another of our lead product candidates, omburtamab;
- approximately \$ \_\_\_\_\_ million to fund our ongoing clinical development of our omburtamab-DTPA product candidate;
- approximately \$ \_\_\_\_\_ million to fund our ongoing clinical development of our BsAb product candidates;
- approximately \$ \_\_\_\_\_ million to fund the additional pre-clinical research and clinical development activity related to our other product candidates and programs; and
- the remainder for working capital and other general corporate purposes, which may include funding for additional research, hiring additional personnel, capital and commercialization expenditures and the costs of operating as a public company.

This expected use of the net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development and commercialization efforts, the status of and results from clinical trials, any collaborations that we may enter into with third parties for our product candidates and any unforeseen cash needs. Moreover, our estimates of the costs to fund our clinical trials are based on the current designs of the trials. If we were to modify the design of any of these clinical trials, for instance, to increase the number of patients in the clinical trials, our costs to fund the clinical trials could increase.

As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

Based on our current plans, we believe that our existing cash and cash equivalents, together with the net proceeds from this offering, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements through February 2020. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. We expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to complete each of our ongoing pivotal stage clinical trials for our lead product candidates, naxitamab and omburtamab. However, we do not expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to conduct through completion any additional clinical trials of our lead product candidates for other indications or to otherwise conduct and complete the development of our other product candidates.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

## **DIVIDEND POLICY**

We have never declared nor paid cash dividends on our common stock. We currently intend to retain all available funds and all of our future earnings, if any, to finance the growth and development of our business. We do not intend to pay cash dividends in respect of our common stock in the foreseeable future. In addition, future debt instruments may materially restrict our ability to pay dividends on our common stock. Payment of future cash dividends, if any, will be at the discretion of the board of directors after taking into account various factors, including our financial condition, operating results, current and anticipated cash needs, the requirements of then-existing debt instruments, if any, and other agreements and other factors the board of directors deems relevant.

## CAPITALIZATION

The following table summarizes our cash and capitalization as of December 31, 2017:

- on an actual basis; and
- on an as adjusted basis to give further effect to (i) our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of \$ \_\_\_\_\_ per share of common stock, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us and (ii) the filing and effectiveness of our amended and restated certificate of incorporation which will occur immediately prior to the completion of this offering.

Our capitalization following the closing of this offering will be adjusted based on the actual initial public offering price and other terms of the offering determined at pricing. You should read this table together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus.

	As of December 31, 2017	
	Actual	As Adjusted
Cash and cash equivalents	\$ _____	\$ _____
Stockholders' equity:		
Common stock, \$0.0001 par value per share, 50,000,000 shares authorized, _____ shares issued and outstanding, actual; 100,000,000 shares authorized, _____ shares issued and outstanding, as adjusted		
Preferred stock, \$0.0001 par value per share, 5,500,000 shares authorized, no shares issued or outstanding, actual; 5,500,000 shares authorized, no shares issued or outstanding, as adjusted		
Additional paid-in capital		
Accumulated and other comprehensive income		
Accumulated deficit	_____	_____
Total stockholders' equity	_____	_____
Total capitalization	\$ _____	\$ _____

The as adjusted information above is illustrative only, and our capitalization following the completion of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. A \$1.00 increase or decrease in the assumed initial public offering price of \$ \_\_\_\_\_ per share of common stock, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders' equity and total capitalization by \$ \_\_\_\_\_ million, assuming that the number of shares of common stock offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase or decrease of 1,000,000 shares of common stock in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase or decrease the as adjusted amount of each of cash and cash equivalents, common stock, par value \$0.0001 per share, additional paid-in capital, total stockholders' equity and total capitalization by \$ \_\_\_\_\_ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The table above is based on the number of outstanding shares of our common stock as of December 31, 2017, and excludes:

- shares of our common stock issuable upon the exercise of stock options outstanding as of December 31, 2017, at a weighted average exercise price of \$            per share; and
- shares of our common stock remaining available for future issuance as of December 31, 2017 under our Amended and Restated 2015 Equity Incentive Plan, or the 2015 Plan.

## DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the public offering price per share of our common stock and the as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book value (deficit) as of December 31, 2017 was \$            million, or \$            per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities, which is not included within stockholders' equity (deficit). Historical net tangible book value (deficit) per share represents our historical net tangible book value (deficit) divided by the            shares of our common stock outstanding as of December 31, 2017.

After giving effect to receipt of the net proceeds from our issuance and sale of            shares of our common stock in this offering at an assumed initial public offering price of \$            per share of common stock, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, and the application of the net proceeds from this offering as described in "Use of Proceeds," our as adjusted net tangible book value as of December 31, 2017 would have been \$            million, or \$            per share. This represents an immediate increase in as adjusted net tangible book value per share of \$            to existing stockholders and immediate dilution of \$            in as adjusted net tangible book value per share to new investors purchasing common stock in this offering.

Dilution per share to new investors is determined by subtracting as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Assumed initial public offering price per share	\$
Historical net tangible book value (deficit) per share as of December 31, 2017	\$ (      )
Increase in as adjusted net tangible book value per share attributable to new investors purchasing shares in this offering	<u>                    </u>
As adjusted net tangible book value per share after this offering	<u>                    </u>
Dilution per share to new investors purchasing shares in this offering	<u><u>                    </u></u>

A \$1.00 increase or decrease in the assumed initial public offering price of \$            per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease our as adjusted net tangible book value by \$            million, our as adjusted net tangible book value per share after this offering by \$            and dilution per share to new investors purchasing shares in this offering by \$           , assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase the as adjusted net tangible book value per share after this offering by \$            and decrease the dilution per share to new investors participating in this offering by \$           , assuming no change in the assumed initial public offering price and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. A decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease the as adjusted net tangible book value per share after this offering by \$            and increase the dilution per share to new investors participating in this offering by \$           , assuming no change in the assumed initial public offering price and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their option to purchase additional shares in full, our as adjusted net tangible book value per share after this offering would be \$ \_\_\_\_\_ per share, representing an immediate increase in as adjusted net tangible book value per share of \$ \_\_\_\_\_ to existing stockholders and immediate dilution in as adjusted net tangible book value per share of \$ \_\_\_\_\_ to new investors purchasing common stock in this offering, assuming an initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If any shares are issued upon exercise of outstanding options, you will experience further dilution.

The following table summarizes as of December 31, 2017, on the as adjusted basis described above, the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and by new investors purchasing shares of common stock in this offering. The calculation below is based on an assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Weighted Average Price Per Share
	Number		Percent		
		%	Amount	Percent	
Existing stockholders		%	\$		%\$
New investors					
<b>Total</b>		<b>100.0%</b>	<b>\$</b>	<b>100.0%</b>	<b>\$</b>

A \$1.00 increase or decrease in the assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the total consideration paid by new investors by \$ \_\_\_\_\_ million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by \_\_\_\_\_ percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by \_\_\_\_\_ percentage points, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. An increase or decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase or decrease the total consideration paid by new investors by \$ \_\_\_\_\_ million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by \_\_\_\_\_ percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by \_\_\_\_\_ percentage points, assuming no change in the assumed initial public offering price.

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to \_\_\_\_\_ % of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors participating in the offering would be increased to \_\_\_\_\_ % of the total number of shares of our common stock outstanding after this offering.

The number of shares purchased from us by existing stockholders is based on \_\_\_\_\_ shares of our common stock outstanding as of December 31, 2017, and excludes:

- \_\_\_\_\_ shares of our common stock issuable upon the exercise of stock options outstanding as of December 31, 2017, at a weighted average exercise price of \$ \_\_\_\_\_ per share; and
- \_\_\_\_\_ additional shares of our common stock remaining available for future issuance as of December 31, 2017 under our 2015 Plan.

Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. New investors will experience further dilution if any of our outstanding options or warrants are exercised, new options are issued and exercised under our equity incentive plans or we issue additional shares of common stock, other equity or convertible debt securities in the future. See the section herein entitled "Risk Factors—If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment."



## SELECTED CONSOLIDATED FINANCIAL DATA

The following tables set forth our selected consolidated financial data for the period indicated. We have derived the consolidated statements of operations data for the years ended December 31, 2016 and 2017 and the consolidated balance sheet data as of December 31, 2016 and December 31, 2017 from our audited consolidated financial statements included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected for any future period. You should read the following selected consolidated financial data together with the more detailed information contained in "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and the related notes included elsewhere in this prospectus.

	<u>Year Ended December 31,</u>	
	<u>2016</u>	<u>2017</u>
	(in thousands, except per share data)	
<b>Consolidated Statement of Operations Data:</b>		
Revenue	\$	\$
Operating expenses:		
Research and development	13,855	
General and administrative	3,184	
Total operating expenses	17,039	
Loss from operations	(17,039)	
Interest and other income (expense)	(18)	
Net loss	\$ (17,057)	\$
Net loss attributable to common stockholders	\$ (17,057)	\$
Net loss per share attributable to common stockholders—basic and diluted <sup>(1)</sup>	\$ (1.21)	\$
Weighted-average common shares outstanding used in computing net loss per share attributable to common stockholders—basic and diluted <sup>(1)</sup>	14,087,456	
As adjusted net loss per share—basic and diluted (unaudited)		\$
As adjusted weighted average common shares outstanding used in computing net loss per share—basic and diluted (unaudited)		

- (1) See Note 4 to our consolidated financial statements included elsewhere in this prospectus for an explanation of the method used to calculate the historical basic and diluted net loss per common share and the weighted average number of shares used in the computation of the per share amounts.

	<u>As of December 31,</u>	
	<u>2016</u>	<u>2017</u>
	(in thousands)	
<b>Consolidated Balance Sheet Data:</b>		
Cash and cash equivalents	\$ 16,875	\$
Working capital <sup>(1)</sup>	14,286	
Total assets	17,261	
Total liabilities	5,200	
Accumulated deficit	(22,400)	
Total stockholders' equity	12,061	

- (1) We define working capital as current assets less current liabilities.

## MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*You should read the following discussion and analysis of our financial condition and results of operations together with our accompanying financial statements and related notes thereto included elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. For convenience of presentation some of the numbers have been rounded in the text below.*

### Overview

We are a late-stage clinical biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer. We have a broad and advanced product pipeline, including two pivotal-stage product candidates—naxitamab and omburtamab—which target tumors that express GD2 and B7-H3, respectively. We are developing naxitamab for the treatment of pediatric patients with R/R high-risk NB, and radiolabeled omburtamab for the treatment of pediatric patients with CNS/LM, from NB. NB is a rare and almost exclusively pediatric cancer that develops in the sympathetic nervous system and CNS/LM is a rare and usually fatal complication of NB in which the disease spreads to the membranes, or meninges, surrounding the brain and spinal cord in the CNS.

We expect to submit a BLA for each of our two lead product candidates in 2018, with a goal of receiving approval by the FDA in 2019. We plan to commercialize both of our lead product candidates in the United States as soon as possible after obtaining FDA approval, if such approval occurs. Additionally, we have two omburtamab follow-on product candidates in pre-clinical development, omburtamab-DTPA and hu-omburtamab, a humanized version of omburtamab, each targeting indications with large adult patient populations. We are also advancing an early-stage, novel pipeline of BsAbs. We believe our BsAbs have the potential to result in improved tumor-binding, longer serum half-life, lower immunogenicity and significantly greater T-cell mediated killing of tumor cells without the need for continuous infusion. Our mission is to become the world leader in developing better and safer antibody-based pediatric oncology products addressing clear unmet medical needs and, as such, have a transformational impact on the lives of patients. We intend to advance and expand our product pipeline into certain adult cancer indications either independently or in collaboration with potential partners.

Since our inception in April 2015, we have devoted substantially all of our resources to organizing and staffing our company, business planning, identifying potential product candidates, conducting pre-clinical studies of our product candidates and clinical trials of our lead product candidates, raising capital, and acquiring and developing our technology platform among other matters. We do not have any products approved for sale and have not generated any revenues from product sales. To date, we have financed our operations primarily through private placements of our securities. We have received aggregate gross proceeds of \$119.6 million through December 31, 2017 from the sale and issuance of our common stock.

As of December 31, 2017, we had an accumulated deficit of \$            million. Our net losses were \$17.1 million and \$            for the years ended December 31, 2016 and 2017, respectively. We have incurred significant net operating losses in every year since our inception and expect to continue to incur increasing net operating losses and significant expenses for the foreseeable future. Our net losses

may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly as we:

- continue to advance our lead product candidates through pivotal stage development towards registration;
- continue to advance our other product candidates through pre-clinical and clinical development;
- continue to identify additional research programs and additional product candidates, as well as additional indications for existing product candidates;
- initiate pre-clinical studies and clinical trials for any additional product candidates we identify;
- develop, maintain, expand and protect our intellectual property portfolio;
- hire additional research, sales force, commercialization, clinical and scientific personnel; and
- incur additional costs associated with operating as a public company, including expanding our operational, finance and management teams.

We believe that our cash on hand together with the net proceeds from this offering will be sufficient to fund our operations through February 2020. We do not expect to generate revenues from product sales unless and until we successfully complete development and obtain regulatory approval for a product candidate, which is subject to significant uncertainty and may never occur. Although no assurance can be given, our goal is to complete the development of our lead product candidates, naxitamab for the treatment of pediatric R/R high-risk NB, and omburtamab for the treatment of CNS/LM from NB, by the end of 2019. Additionally, we currently use CROs, and contract manufacturing organizations, or CMOs, to carry out our pre-clinical and clinical development activities and we do not yet have a sales organization.

Moreover, pursuant to the MSK License, we have obtained exclusive rights to MSK's rights in our current product candidates. Under the MSK License, we have committed to funding scientific research and conducting certain clinical trial activities at MSK through 2020. As these product candidates progress through clinical development, regulatory approval and commercialization, certain milestone payments will come due either as a result of the milestones having been met or the passage of time even if the milestones have not been met. Also, we will owe MSK customary royalties on commercial sales of our approved products, including a fixed minimum royalty starting in 2020 whether or not product sales are ever achieved. In addition, we have committed to obtain certain personnel and laboratory services at MSK under our MDSA, and two separate CFSAs. Also under our MCTA with MSK, we will provide drug product and funding for certain clinical trials at MSK. These MSK agreements are important to our business. For a more detailed discussion of the terms and conditions of these agreements, see the section herein entitled "Business—Intellectual Property—MSK Agreements."

If we obtain regulatory approval for our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, we may continue to fund our operations through public or private equity or debt financings or other sources, including strategic collaborations. We may, however, be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our current product candidates, or any additional product candidates, if developed. Because of the numerous risks and uncertainties associated with the development of our existing product candidates and any future product candidates, our platform and technology and because the extent to which we may enter into collaborations with

third parties for development of any of our product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. If we raise additional funds through collaborations, strategic alliances, or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs, product candidates or grant licenses on terms that may not be favorable to us and could have a negative impact on our financial condition.

## **Components of Our Results of Operations**

### ***Revenue***

To date, we have not generated any revenue from product sales and do not expect to do so in the near future. We expect that we will experience increasing losses as we continue our development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. Our ability to generate revenue for each product candidate for which we receive regulatory approval, if any, will depend on numerous factors, including reimbursement, coverage, competition, commercial manufacturing capability and market acceptance of such approved products.

### ***Operating Expenses***

#### ***Research and Development Expenses***

Research and development expenses consist of expenses incurred in connection with the discovery and development of our product candidates. We expense research and development costs as incurred. These expenses include:

- sponsored research, laboratory facility services, clinical trial and data service at MSK under our SRA, our two CFSAs, our MCTA, and our MDSA, with MSK;
- expenses incurred under agreements with CROs, as well as investigative sites and consultants that conduct our non-clinical studies and pre-clinical and clinical trials;
- expenses incurred under agreement with CMOs, including manufacturing scale-up expenses and the cost of acquiring and manufacturing pre-clinical and clinical trial materials, including manufacturing validation batches;
- upfront and milestone and other payments due under our third-party licensing agreements;
- employee-related expenses, which include salaries, benefits, travel and stock-based compensation;
- expenses relating to regulatory activities, including filing fees paid to regulatory agencies;
- outsourced professional scientific development services; and
- allocated expenses for utilities and other facility-related costs, including rent, insurance, supplies and maintenance expenses, and other operating costs.

The successful development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of, or when, if ever, material net cash inflows may commence from naxitamab and omburtamab or any future product candidates we may develop. This uncertainty is due to the numerous risks and uncertainties associated with the duration and cost of clinical trials, which vary significantly over the life of a project as a result of many factors, including:

- the number of clinical sites included in the trials;

- the availability and length of time required to enroll a sufficient number of suitable patients in our clinical trials;
- the actual probability of success for our product candidates, including the safety and efficacy, early clinical data, competition, manufacturing capability and commercial viability;
- significant and changing government regulation and regulatory guidance;
- the performance of our existing and any future collaborators;
- the number of doses patients receive;
- the duration of patient follow-up;
- the results of our clinical trials and pre-clinical studies;
- the establishment of commercial manufacturing capabilities;
- adequate ongoing availability of raw materials and drug substance for clinical development and any commercial sales;
- the receipt of marketing approvals, including a safety, tolerability and efficacy profile that is satisfactory to the FDA or any non-U.S. regulatory authority;
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the commercialization of approved products.

Our expenditures are subject to additional uncertainties, including the terms and timing of regulatory approvals. We may never succeed in achieving regulatory approval for naxitamab, omburtamab or any other product candidates we may develop.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials beyond those that we anticipate will be required for the completion of clinical development of a product candidate, or if we experience significant delays in our clinical trials due to patient enrollment or other reasons, we would be required to expend significant additional financial resources and time on the completion of clinical development.

Research and development activities are central to our business model. Product candidates in later stages of clinical development, like naxitamab and omburtamab, generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect our research and development expenses to increase significantly over the next several years as we increase personnel costs, including stock-based compensation, conduct clinical trials and potentially prepare regulatory filings for naxitamab and omburtamab.

#### ***General and Administrative Expenses***

General and administrative expenses consist primarily of employee related expenses, including salaries, bonus, benefits, and stock-based compensation expenses for personnel in executive, finance and administrative functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to corporate matters, and fees for patent, accounting, tax, and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, potential commercialization of our product

candidates and increased costs associated with operating as a public company, including expenses related to services associated with maintaining compliance with exchange listing and the SEC requirements, regulatory expenses, director and officer insurance costs and investor and public relations costs. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, if and when we believe a regulatory approval of a product candidate appears likely, we anticipate an increase in payroll and other employee-related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of that product candidate.

### **Critical Accounting Policies and Significant Judgments and Estimates**

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles, or GAAP. We believe that several accounting policies are significant to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this prospectus, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

#### ***Research and Development Expenses***

Research and development costs are charged to operations when incurred and are included in operating expenses. Research and development costs consist principally of compensation cost for our employees and consultants that perform our research activities, the fees paid to maintain our licenses, the payments to third parties for manufacturing and clinical research organizations and additional product development, and consumables and other materials used in research and development. We record accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, we analyze progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. Actual results could differ from our estimates. We are obligated to make certain milestone and royalty payments in accordance with the contractual terms of the MSK License based upon the resolution of certain contingencies. Certain of these milestone payments are due and payable with the passage of time whether or not the milestones have actually been met. We record the milestone and royalty payment when the achievement of the milestone (including the passage of time) or payment of the milestone or royalty is probable and the amount of the payment is reasonably estimable.

#### ***Use of Estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these financial statements include, but are not limited to, the accrual for research and development expenses, the accrual of milestone and royalty payments, the valuation of shares of common stock and stock options.

Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

### ***Income Taxes***

We account for income taxes under the asset and liability approach for the financial accounting and reporting of income taxes. Deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to net operating loss carry forwards and temporary differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. These assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which the temporary differences are expected to reverse. A valuation allowance is established when management determines that it is more likely than not that some portion or all of the deferred tax assets will not be realized.

We prepare and file tax returns based on its interpretation of tax laws and regulations. In the normal course of business, our tax returns are subject to examination by various taxing authorities. Such examinations may result in future tax and interest assessments by these taxing authorities. In determining our tax provision for financial reporting purposes, we establish a reserve for uncertain tax positions unless such positions are determined to be more likely than not of being sustained upon examination based on their technical merits. We consider many factors when evaluating and estimating our tax positions and tax benefits, which may require periodic adjustments and which may not accurately anticipate actual outcomes. Accordingly, we will report a liability for unrecognized tax benefits resulting from any uncertain tax positions taken or expected to be taken on a tax return.

Our policy is to recognize, when applicable, interest and penalties on uncertain tax positions as part of income tax expense.

### ***Stock-Based Compensation***

We measure stock options granted to employees and directors based on the fair value on the date of the grant and recognize compensation expense of those awards, over the requisite service period, which is the vesting period of the respective award. Forfeitures are accounted for as they occur. We issue stock options with only service-based vesting conditions and record the expense for these awards using the straight-line method over the requisite service period.

For share-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of our shares of common stock and updated assumption inputs in the Black-Scholes option-pricing model.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model. Historically, we have been a private company and lack company-specific historical and implied volatility information for our shares. Therefore, we estimate our expected share price volatility based on the historical volatility of a group of publicly-traded peer companies and we expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded share price. The expected term of our stock options has been determined utilizing the "simplified" method for awards as we have limited historical data to support the expected term assumption. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield is based on the fact that we have never paid cash dividends on shares of our common stock and do not expect to pay any cash dividends in the foreseeable future.

### ***Determination of the Fair Value of Common Stock***

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each award grant, with input from management, considering our most recently completed or ongoing private placement activities, which resulted in estimated fair value of our common stock of \$0.20 per share as of June 6, 2015; \$4.38 per share as of May 20, 2016; \$4.38 per share as of October 21, 2016; \$4.38 per share as of August 22, 2016; \$8.50 per share as of December 14, 2016; \$9.35 per share as of September 13, 2017; and \$9.35 per share as of December 5, 2017. In addition to considering the results of such recently completed or ongoing private placements, our board of directors considered various objective and subjective factors to determine the fair value of our common stock as of each grant date including:

- the progress of our research and development programs, including the status of pre-clinical studies and clinical trials for our product candidates;
- our stage of development and commercialization and our business strategy;
- material risks related to our business;
- external market conditions affecting the biotechnology industry, and trends within the biotechnology industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our common stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or a sale of our company in light of prevailing market conditions; and
- an analysis of IPOs and the market performance of similar companies in the biopharmaceutical industry.

The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different.

Following the completion of this offering, the fair value of our common stock will be determined based on the quoted market price of our common stock.

#### ***Stock Options Granted***

The following table sets forth by grant date the number of shares of common stock subject to options granted in 2016, the per share exercise price of the options, the fair value per share of common stock on each grant date, and the per share estimated fair value of the options:

<b>Grant Date</b>	<b>Type of Award</b>	<b>Number of Shares</b>	<b>Per Share Exercise Price</b>	<b>Estimated Fair Value Per Share on Grant Date</b>
May 20, 2016	Option	220,000	\$ 4.38	\$ 2.66
August 22, 2016	Option	20,000	\$ 4.38	\$ 2.58
October 21, 2016	Option	571,000	\$ 4.38	\$ 2.63
December 14, 2016	Option	48,000	\$ 8.50	\$ 5.26



**Fair Value of Stock Options**

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model and the following assumptions, presented on a weighted average basis:

	Year Ended December 31,		
	2016	2017	
Expected volatility	60.60%		%
Risk-free interest rate	1.77%		%
Expected term (in years)	7.00		
Expected dividend yield	0.00%		%

- Risk-free interest rate: The risk-free interest rate assumption is based on the U.S. Treasury instruments whose terms were consistent with the expected option term of our stock options.
- Expected Dividend Yield: The expected dividend yield assumption is based on the fact that we have never paid cash dividends and have no present intention to pay cash dividends. Consequently, we used an expected dividend of zero.
- Expected Volatility: The expected stock price volatility is estimated by taking the average historic price volatility of industry peers and adjusting for differences in our life cycle and financing leverage. Our industry peers consist of several public companies in the biopharmaceutical industry.
- Expected life: We determine the average expected life of stock options based on the simplified method in accordance with SEC Staff Accounting Bulletin Nos. 107 and 110, as our common stock to date has not been publicly traded. We expect to continue to use the simplified method until we have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term.

**Results of Operations****Comparison of the Years Ended December 31, 2016 and 2017**

The following table summarizes our results of operations for the years ended December 31, 2016 and 2017:

	Year Ended December 31,		Change
	2016	2017	
	(in thousands)		
Operating expenses:			
Research and development	\$ 13,855	\$	
General and administrative		3,184	
Total operating expenses		17,039	
Loss from operations		(17,039)	
Interest and other income (expense)		(18)	
Net loss	\$ (17,057)	\$	

**Research and Development Expenses**

Research and development expenses by \$ million, from \$13.9 million for the year ended December 31, 2016, to \$ million for the year ended December 31, 2017. This was primarily due to clinical and manufacturing activities. The expenses resulted from a \$ million in CMO expenses for our lead product candidates, naxitamab and omburtamab. CRO expenses by \$ million and expenses to MSK incurred under our SRAs, CFSAs, MCTA and MDSA by \$ million. Employee-related costs including salary, benefits and non-cash stock-based compensation for personnel related to our business activities, by \$ for the year ended December 31, 2017. These were partly offset by a \$ million in patent expenses, primarily caused by a of historic patent expenses to MSK.

**General and Administrative Expenses**

General and administrative expenses by \$ million, from \$3.2 million for the year ended December 31, 2016, to \$ million for the year ended December 31, 2017. The in general and administrative expenses was primarily attributable to a \$ million in employee-related costs, including salary, benefits and non-cash stock-based compensation for personnel related to our business activities. Travel expenses and rent of premises expenses were \$ million for the year ended December 31, 2016, as compared to \$ million for the year ended December 31, 2017, as a result of our business activities in 2017.

**Interest and Other Income (Expense)**

Other expenses for the year ended December 31, 2016 were \$18,000 as compared to other expenses of \$ for the year ended December 31, 2017. Our interest income has not been significant due to low investment balances and low interest earned on those balances.

**Liquidity and Capital Resources****Overview**

Since our inception we have incurred significant net operating losses and expect to continue to incur increasing net operating losses and significant expenses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We do not currently have any approved products and have never generated any revenue from product sales. We have financed our operations through December 31, 2017 primarily through gross proceeds of \$119.6 million from the sale of our common stock. As of December 31, 2017, we had cash and cash equivalents of \$ million. We will need additional capital to continue funding our operations, which we may obtain from additional equity or debt financings, collaborations, licensing arrangements, or other sources.

**Cash Flows**

The following table provides information regarding our cash flows for the years ended December 31, 2016 and December 31, 2017:

	Year Ended December 31,	
	2016	2017
	(in thousands)	
Cash used in operating activities	\$ (11,166)	\$ ( )
Cash used in investing activities	—	( )
Cash provided by financing activities	18,972	
Net increase in cash and cash equivalents	<u>\$ 7,806</u>	<u>\$</u>

### Net Cash Used in Operating Activities

The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital.

Net cash used in operating activities was \$11.2 million during the year ended December 31, 2016, as compared to \$ million during the year ended December 31, 2017. The in net cash used in operations was primarily due to in our net loss of \$ million for the year ended December 31, 2017, as compared to \$17.1 million for the year ended December 31, 2016. This was primarily due to in our operating expenses in connection with development of our lead product candidates, naxitamab and omburtamab, and the expansion of our other business activities. The was partially offset by non-cash stock-based compensation to employees, which by \$ million. Adjustment of working capital reflects in accrued expenses, accounts payable, and other non-current liabilities of \$ million and in other assets of approximately \$ million for the year ended December 31, 2017, as compared to the year ended December 31, 2016.

### Net Cash Used in Investing Activities

We did not use cash for investing activities in the years ended December 31, 2016 and 2017.

### Net Cash Provided by Financing Activities

Net cash provided by financing activities was \$19.0 million during the year ended December 31, 2016, as compared to \$ million during the year ended December 31, 2017. The in cash provided by financing activities was attributable to net proceeds of \$ million related to the issuance of common stock in a series of private placements in the year ended December 31, 2017.

### Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we complete clinical development of our lead product candidates, naxitamab and omburtamab, and potentially initiate our planned BLA submissions for both product candidates. In addition, we plan to advance the development of other pipeline programs, initiate new research and pre-clinical development efforts and seek marketing approval for any additional product candidates that we successfully develop. If we obtain marketing approval for any of our product candidates, we expect to incur commercialization expenses, which may be significant, related to establishing sales, marketing, manufacturing capabilities, distribution and other commercial infrastructure to commercialize such products. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we might need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs and/or future commercialization efforts.

We believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents as of December 31, 2017, will enable us to fund our operating expenses and capital expenditure requirements through February 2020.

We have based our projections of operating capital requirements on assumptions that may prove to be incorrect and we may use all of our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with the development and commercialization of naxitamab and omburtamab, and the research, development and commercialization of other potential product candidates, we are unable to estimate the exact amount of

our operating capital requirements. Our future capital requirements will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials for developing our lead product candidates, naxitamab and omburtamab, and conducting pre-clinical studies and clinical trials for our other product candidates;
- research and pre-clinical development efforts for any future product candidates that we may develop;
- our ability to enter into and the terms and timing of any collaborations, licensing agreements or other arrangements;
- the achievement of milestones or occurrence of other developments that trigger payments under any collaboration or other agreements;
- the number of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of our current and future product candidates;
- proceeds received, if any, from monetization of any future PRVs;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting pre-clinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest in our company may be materially diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be

favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

### Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under the applicable regulations of the SEC.

### Contractual Obligations and Commitments

The following table summarizes our contractual obligations as of December 31, 2017 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments Due by Period				
	Total	Less Than 1 Year	1 to 3 Years (in thousands)	4 to 5 Years	More Than 5 Years
Operating lease commitments(1)	\$	\$	\$	\$	\$
Collaboration funding(2)					
<b>Total contractual obligations</b>	<b>\$</b>	<b>\$</b>	<b>\$</b>	<b>\$</b>	<b>\$</b>

- (1) Reflects payments due for our leases of office space under operating lease agreements that expire in 2023.
- (2) Reflects non-cancellable fees due in connection with our agreements with MSK as of December 31, 2017.

We enter into contracts in the normal course of business with CROs, CMOs, clinical sites and other third parties for clinical trials, pre-clinical research studies and testing, professional consultants for expert advice and other vendors for clinical supply, manufacturing and other services. These contracts are not considered contractual obligations, as they provide for termination upon prior notice, and, therefore, are cancelable contracts and do not include any minimum purchase commitments. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including non-cancellable obligations of our service providers, up to the date of cancellation. These payments are not included in the table of contractual obligations and commitments above.

As further described below, under various licensing and related agreements with third parties, we have agreed to make milestone and royalty payments to third parties. We have not included the contingent payment of certain milestones in the table above, which timing cannot be determined because they are not date certain. In addition, we have other contingent payment obligations, such as such as royalties or other third party milestones, which are not included in the table above as the amount, timing and likelihood of such payments are not known.

Under the MSK License and the MSK CD33 License, we are obligated to (i) make certain payments to MSK, which become due based upon the achievement of the related milestone activities or the passage of time in the event such milestone activities are not achieved, as well as certain sales-related milestones, (ii) pay mid to high single-digit royalties to MSK, on a product-by-product and country-by-country basis, of a mid-to-high single-digit royalties based on net sales of products licensed under the applicable agreement and (iii) pay to MSK a percentage of any sublicense fees received by us. We are also required to pay annual minimum royalties of \$80,000 over the royalty term, starting in 2020. These amounts are non-refundable but are creditable against royalty payments otherwise due under the MSK License. Total expensed minimum royalty payments under the MSK License were

\$1,200,000 in 2016 and \$            in 2017, all of which were recorded as long-term accrued liabilities as of December 31, 2016 and December 31, 2017, respectively, upon determination that the payment of such minimum royalties was probable and the amount was estimable. We are also obligated to pay MSK certain clinical, regulatory and sales-based milestone payments under the MSK License. These milestone payments become due upon achievement of the related clinical, regulatory or sales-based milestone defined in the MSK License. Certain of the clinical and regulatory milestone payments become due at the earlier of completion of the related milestone activity or the date indicated in the MSK License, whether or not the milestone activity is achieved. Total clinical and regulatory milestones potentially due under the MSK License are \$2,450,000 and \$9,000,000, respectively. There are also sales-based milestones that become due should the Company achieve certain amounts of sales of licensed products resulting from the MSK License, with total sales-based milestones potentially due of \$20,000,000. The Company records milestones in the period in which the contingent liability is probable and the amount is reasonably estimable. In addition, to the extent we enter into sublicense arrangements, we are required to pay to MSK a percentage of certain payments that we receive from sublicensees of the rights licensed to us by MSK, which percentage will be based upon the date we receive such payments or the achievement of certain clinical milestones. To date, we have not entered into any sublicenses related to the MSK License. For a more detailed discussion of these payments, see the section herein entitled "Business—Intellectual Property—MSK Agreements." As of December 31, 2017, we were unable to estimate the timing or likelihood of achieving the milestones or generating future product sales and therefore, any related payments are not included in the table above.

In addition, under the MSK CD33 License, we are required to pay total potential milestone payments of \$550,000, \$500,000 and \$7,500,000 for clinical, regulatory and sales based milestones, respectively. We are required to pay royalty payments that become due beginning in year 10 of \$40,000 per year prior, subject to increase and creditable against any royalty payments due based on sales in the future. We are also required to pay mid to high single-digit royalties on sales of licensed products. Additionally, we have agreed to pay to MSK approximately \$1,360,000 for research services related to the intellectual property licensed by us under the MSK CD33 License. The research services are expected to occur over the two year period immediately following the MSK CD33 License.

### **Recent Accounting Pronouncements**

Refer to Note 3, "Summary of Significant Accounting Policies," in the accompanying notes to the consolidated financial statements for a discussion of recent accounting pronouncements.

### **Internal Controls and Procedures**

We will be required, pursuant to Section 404(a) of the Sarbanes-Oxley Act, or Section 404, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting for the year following our first annual report required to be filed with the SEC. This assessment will need to include disclosure of any material weaknesses identified by management over our internal control over financial reporting. However, our independent registered public accounting firm will not be required to report on the effectiveness of our internal control over financial reporting pursuant to Section 404(b) until the later of the year following our first annual report required to be filed with the SEC, or the date we are no longer an "emerging growth company" if we take advantage of the exemptions contained in the JOBS Act.

Internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with GAAP. Prior to this offering, we were a private company and we are currently planning a process for reviewing, documenting and testing our internal control over financial reporting. Certain material weaknesses have been identified in our internal control over financial reporting. See the section herein entitled "Risk Factors—It has been determined that we have material weaknesses in our internal

control over financial reporting. If our remediation of these material weaknesses is not effective, or if we experience additional material weaknesses or otherwise fail to maintain an effective system of internal controls in the future, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, the value of our common stock. In addition, because of our status as an emerging growth company, our independent registered public accounting firm is not required to provide an attestation report as to our internal control over financial reporting for the foreseeable future." If we fail to maintain an effective system of disclosure controls and internal control over financial reporting, our ability to produce timely and accurate financial statements or comply with applicable regulations could be impaired. If we are unable to remediate these identified material weaknesses, or if we experience additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations, or comply with the accounting and reporting requirements applicable to public companies, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

We have not performed an evaluation of our internal control over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act, nor have we engaged an independent registered public accounting firm to perform an audit of our internal control over financial reporting as of any balance sheet date or for any period reported in our financial statements. Presently, we are not an accelerated filer, as such term is defined by Rule 12b-2 of the Exchange Act, and therefore, our management is not presently required to perform an annual assessment of the effectiveness of our internal control over financial reporting. This requirement will first apply to our second Annual Report on Form 10-K. Our independent public registered accounting firm will first be required to attest to the effectiveness of our internal control over financial reporting for our Annual Report on Form 10-K for the first year we are no longer an "emerging growth company."

We are in the very early stages of the costly and challenging process of compiling the system and processing documentation necessary to perform the evaluation needed to comply with Section 404. We may not be able to complete our evaluation, testing or any required remediation in a timely fashion. During the evaluation and testing process, if we identify one or more material weaknesses in our internal control over financial reporting, we will be unable to assert that our internal controls are designed and operating effectively, which could result in a loss of investor confidence in the accuracy and completeness of our financial reports. This could cause the price of our common stock to decline, and we may be subject to investigation or sanctions by the SEC.

### **Emerging Growth Company Status; The JOBS Act**

The JOBS Act permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

For so long as we are an emerging growth company we expect that:

- we will present in this prospectus only two years of audited financial statements, in addition to any required unaudited financial statements, with correspondingly reduced Management's Discussion and Analysis of Financial Condition and Results of Operations disclosure;
- we will avail ourselves of the exemption from the requirement to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding a supplement to the auditor's report providing additional information about the audit and the financial statements;

- we will avail ourselves of the exemption from the requirement to obtain an attestation and report from our auditors on the assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act; and
- we will provide less extensive disclosure about our executive compensation arrangements.

We will remain an emerging growth company for up to five years, although we will cease to be an "emerging growth company" upon the earliest of: (i) the last day of the fiscal year following the fifth anniversary of this offering, (ii) the last day of the first fiscal year in which our annual gross revenues are \$1.07 billion or more, which amount is periodically updated, (iii) the date on which we have, during the previous rolling three-year period, issued more than \$1.0 billion in non-convertible debt securities or (iv) the date on which we are deemed to be a "large accelerated filer" as defined in the Exchange Act.

## **Qualitative and Quantitative Disclosures About Market Risk**

### ***Interest Rate Risk***

We are exposed to market risk related to changes in interest rates. As of December 31, 2016 and December 31, 2017, we had cash and cash equivalents of \$16.9 million and \$            million, respectively, maintained primarily with financial institutions in federally insured accounts and held in an unrestricted escrow account. We currently have, and may, from time to time in the future, cash in banks in excess of FDIC insurance limits. We have not experienced any losses to date resulting from this practice. We mitigate our risk by maintaining the majority of our cash and equivalents with high quality financial institutions. Our exposure to changes in the general level of U.S. interest rates is considered immaterial, particularly because our cash equivalents are primarily held in day-to-day bank accounts. Due to short-term nature of such balances, an immediate 100 basis point change in interest rates would not have any effect on the fair market value of cash balances.

### ***Foreign Currency Exchange Risk***

Our primary exposure to market risk is foreign exchange rate sensitivity to the Danish Kroner (DKK), the currency used in the Kingdom of Denmark, where our wholly owned subsidiary, Y-mAbs Therapeutics A/S, is located. As of December 31, 2016 and December 31, 2017, we had cash and cash equivalents denominated in DKK of \$0.6 million and \$            million, respectively, and an immediate 5% change in DKK exchange rate would not have any material effect on the fair market value of cash balances with the subsidiary.



## BUSINESS

### Overview

We are a late-stage clinical biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer. We have a broad and advanced product pipeline, including two pivotal-stage product candidates—naxitamab and omburtamab—which target tumors that express GD2 and B7-H3, respectively. We are developing naxitamab for the treatment of pediatric patients with relapsed or refractory, or R/R, high-risk neuroblastoma, or NB, and radiolabeled omburtamab for the treatment of pediatric patients with central nervous system, or CNS, leptomeningeal metastases, or LM, from NB. NB is a rare and almost exclusively pediatric cancer that develops in the sympathetic nervous system and CNS/LM is a rare and usually fatal complication of NB in which the disease spreads to the membranes, or meninges, surrounding the brain and spinal cord in the CNS.

We expect to submit a Biologics License Application, or BLA, for each of our two lead product candidates in 2018, with a goal of receiving approval by the U.S. Food and Drug Administration, or FDA, in 2019. We plan to commercialize both of our lead product candidates in the United States as soon as possible after obtaining FDA approval, if such approval occurs. We have two additional omburtamab follow-on product candidates in pre-clinical development, omburtamab-DTPA and hu-omburtamab, a humanized version of omburtamab, each targeting indications with large adult patient populations. We are also advancing an early-stage, novel pipeline of bispecific antibodies, or BsAbs. We believe our BsAbs have the potential to result in improved tumor-binding, longer serum half-life, lower immunogenicity and significantly greater T-cell mediated killing of tumor cells without the need for continuous infusion. Our mission is to become the world leader in developing better and safer antibody-based pediatric oncology products addressing clear unmet medical needs and, as such, have a transformational impact on the lives of patients. We intend to advance and expand our product pipeline into certain adult cancer indications either independently or in collaboration with potential partners.

Naxitamab is a recombinant humanized immunoglobulin G, or IgG1k, monoclonal antibody that targets ganglioside GD2, which is highly expressed in various neuroectoderm-derived tumors and sarcomas. Naxitamab is currently being studied in several clinical trials, including pivotal-stage development (Study 201) and a Phase 1/2 clinical trial (Study 12-230) for the treatment of pediatric R/R high-risk NB, a Phase 2 clinical trial (Study 16-1643) in front-line NB and a Phase 2 clinical trial (Study 15-096) for relapsed osteosarcoma. We believe that naxitamab has multiple potential advantages over other GD2-targeting antibody-based therapies. In particular, its modest toxicity allows for doses two-and-a-half times greater than existing GD2-targeting antibody-based therapies. Unlike currently approved GD2-targeting therapies for NB, which require 10 to 20 hours of infusion and hospitalization for several days, naxitamab is administered in approximately 30 minutes in an outpatient setting. We believe this significantly shorter administration time is an important advantage considering the overall pain associated with treatment.

In Study 12-230 for naxitamab, which together with Study 201 is expected to form the primary basis of our BLA submission, we achieved an overall response rate, or ORR, of 57% in 23 patients with pediatric R/R high-risk NB who at study entry had evaluable tumors and no evidence of progression of disease, or PD. Based on our discussions with the FDA, the profile of the non-PD R/R high-risk NB pediatric patients in Study 12-230 is representative of the intended patient population for naxitamab's target indication. The corresponding ORRs will form the primary objective of our pivotal study (Study 201). Additionally, based on our discussions with the FDA, we believe that naxitamab may qualify for accelerated approval if we can demonstrate a 30% ORR (which is significantly different from a 20% ORR at a 95% confidence interval, or CI) with a minimum 12-week duration of response. We have proposed to the FDA that, pending comparability between the study population and the

pharmacokinetics analysis in Study 12-230 and Study 201, the data from the two studies may be pooled for analysis. Naxitamab has been administered to more than 200 patients to date, who will form the safety portion of our planned BLA submission, and has been generally well tolerated with no significant long-term toxicities observed to date. We expect to report the topline results from our ongoing Study 201 and submit the BLA for naxitamab for R/R high-risk NB in the second half of 2018. Currently, there are no FDA-approved therapies for primary refractory or second-line pediatric NB patients. Naxitamab has also received orphan drug designation, or ODD, and rare pediatric disease designation, or RPDD, from the FDA for the treatment of NB. While our current clinical efforts for naxitamab are focused on rare pediatric cancers, we believe that we can potentially expand its application to the treatment of adults with cancers that express GD2. We estimate that there were more than 200,000 new adult patients diagnosed with GD2-positive cancers in the United States in 2017.

Omburtamab is a murine monoclonal antibody that targets B7-H3, an immune checkpoint molecule that is widely expressed in tumor cells of several cancer types. <sup>131</sup>I-omburtamab, which is omburtamab radiolabeled with Iodine-131, is currently being studied in several clinical trials including pivotal-stage development (Study 101) and a Phase 1 clinical trial (Study 03-133) for the treatment of pediatric patients with R/R NB who have CNS or LM. As of August 2017, 93 patients with pediatric CNS/LM from NB had been treated with <sup>131</sup>I-omburtamab in Study 03-133. An analysis of these 93 patients demonstrated a median overall survival, or OS, of 47 months (including an estimated five-year OS of approximately 43%), as compared to historical median OS of approximately six months. We have proposed to the FDA that, pending comparability between study population and the pharmacokinetics analysis in Study 03-133 and Study 101, data from both studies may be pooled for analysis for our planned BLA submission. Omburtamab has also been generally well tolerated, with no significant long-term toxicities observed to date. <sup>131</sup>I-omburtamab has received ODD and RPDD from the FDA for the treatment of NB, and Breakthrough Therapy Designation, or BTD, for the treatment of pediatric patients with R/R NB who have CNS or LM. We expect to submit the BLA for <sup>131</sup>I-omburtamab for CNS/LM from NB in the second half of 2018.

<sup>124</sup>I-omburtamab, which is omburtamab radiolabeled with Iodine-124, is currently being studied for the treatment of Diffuse Intrinsic Pontine Glioma, or DIPG. <sup>131</sup>I-omburtamab is currently being studied for the treatment of Desmoplastic Small Round Cell Tumors, or DSRCT. Both DIPG and DSRCT are rare, and often fatal, cancers. While our current clinical efforts are focused on rare pediatric cancers, we believe we can potentially expand omburtamab's application to the treatment of CNS/LM resulting from other adult and pediatric solid tumors expressing B7-H3 and the underlying solid systemic tumors. We estimate that, in the United States in 2017, there were more than 30,000 new patients diagnosed with cancer that has metastasized to the CNS/LM, of which the vast majority express B7-H3.

We have two additional product candidates in pre-clinical development, omburtamab-DTPA (diethylenetriamine pentaacetate), a Lutetium-177 conjugated antibody, and hu-omburtamab, a humanized version of omburtamab, each targeting indications with large adult patient populations where we believe there is a significant unmet medical need. We are also advancing a pipeline of novel BsAbs through late pre-clinical development, including our naxitamab-BsAb product candidate for the treatment of refractory GD2-positive adult and pediatric solid tumors and a huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. In pre-clinical studies, naxitamab-BsAb has demonstrated the potential for improved tumor-binding, longer serum half-life, lower immunogenicity and significantly greater T-cell mediated killing compared to existing bispecific constructs. We expect to file an Investigational New Drug application, or IND, for our naxitamab-BsAb product candidate for treatment of patients with refractory GD2-positive solid tumors in the first half of 2018.

We have exclusive rights to MSK's rights in all of our current product candidates under our 2015 license agreement, or the MSK License, with Memorial Sloan Kettering Cancer Center, or MSK.

The MSK License also provides us with non-exclusive access to technology that involves the creation of a novel human protein tag that can potentially dimerize, or link together, bispecific T-cell engagers, or BiTEs. We refer to this technology as the MULTI-TAG technology. We plan to create a broad platform of dimerized BiTEs using the MULTI-TAG technology and are currently collaborating with MSK on several MULTI-TAG product candidates, with the goal of selecting a potential clinical candidate in 2018. We believe that our strong relationship with MSK, one of the world's leading cancer treatment centers, and our access to certain of MSK's technologies and substantial research capabilities affords us several competitive advantages, particularly with respect to patient recruitment for clinical trials. Under a separate 2017 CD33 license agreement with MSK, or the MSK CD33 License, we have a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make, and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments in connection with certain CD33 antibodies developed in the laboratory of a specific principal investigator at MSK and constructs thereof.

Our management team has substantial public company experience and extensive knowledge in the field of antibody oncology drug development, manufacturing and commercialization. Thomas Gad, our Founder, Chairman, President and Head of Business Development, co-founded Singad Pharma ApS, a Danish pharmaceutical and distribution company, where, as part of senior management, he gained more than 12 years of experience in the pharmaceutical industry, including in business development, financing and licensing negotiations and manufacturing site qualification. Our Chief Executive Officer, Dr. Claus Juan Møller San Pedro, co-founded Genmab A/S, one of the largest public biotechnology companies in Europe, where he served as Executive Vice President and Chief Operating Officer for over 10 years. Our Chief Financial Officer, Bo Kruse, served as Genmab's Chief Financial Officer and was directly involved in several of Genmab's financing rounds including Genmab's initial public offering. Our Senior Vice President and Chief Operating Officer, Joris Wiel Jan Wilms, has extensive industry experience in clinical development, primarily within oncology and hematology indications, and was responsible for overseeing several first-in-human studies and pivotal clinical trials, leading to the approval of two monoclonal antibody-based products while at his previous positions as Vice President—Clinical Trial Services and Pharmacovigilance at KLIFO A/S, and Associate Director of Clinical Development at Genmab. Our Senior Vice President of Technical Operations, Dr. Torben Lund-Hansen, has substantial experience in antibody process development and manufacturing. Dr. Lund-Hansen held similar positions at Genmab where he was responsible for sourcing clinical and commercial drug substance and product manufacturing. Our Chief Medical Officer, Dr. Steen Lisby, also comes from Genmab where he was Genmab's Chief Medical Officer until July 2017 when he joined our company. Dr. Lisby also has substantial experience in antibody drug development. In addition, since our inception in April 2015, we have raised approximately \$120 million from our founding investors and prominent biotechnology institutional investors, including HBM Healthcare Investments (Cayman) Ltd. and funds advised by or affiliated with Scopia Capital Management LP and Sofinnova Ventures, Inc., among others, and as of \_\_\_\_\_, 2018, we have cash and cash equivalents of \$ \_\_\_\_\_.

## Our Pipeline

The following table sets forth our product candidates and their current development stages, estimated development timelines and anticipated milestones.

Product Candidate	Target	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Next Anticipated Milestone(s)
Naxitamab	GD2	Relapsed (Second-Line) / Refractory High-Risk Neuroblastoma (Pediatric)	Ongoing pivotal trial <sup>(1)</sup>				<ul style="list-style-type: none"> <li>• 2H 2018 – Announce Phase 3 topline data</li> <li>• 2H 2018 – BLA submission</li> <li>• 2019 – Potential FDA approval</li> </ul>
		(Front-Line) Consolidation of 1 <sup>st</sup> Remission High-Risk Neuroblastoma (Pediatric)	Ongoing Phase 2 trial				• 2019 – Announce topline data
		Relapsed (Second-Line) Osteosarcoma <sup>(2)</sup>	Ongoing Phase 2 trial				• 2019 – Announce topline data
Omburtamab	B7-H3	CNS / Leptomeningeal Metastases from Neuroblastoma (Pediatric) ( <sup>131</sup> I) <sup>(3)</sup>	Ongoing pivotal trial <sup>(4)</sup>				<ul style="list-style-type: none"> <li>• 2H 2018 – BLA submission</li> <li>• 2019 – Potential FDA approval</li> </ul>
		Diffuse Intrinsic Pontine Glioma (Pediatric) ( <sup>131</sup> I) <sup>(3)</sup>	Ongoing Phase 1 trial				• 1H 2018 – Announce Phase 1 topline data
		Desmoplastic Small Round Cell Tumor (Pediatric) ( <sup>131</sup> I) <sup>(3)</sup>	Ongoing Phase 1 trial				• 1H 2018 – Announce Phase 1 topline data
Omburtamab-DTPA <sup>(5)</sup>	B7-H3	B7-H3 Positive CNS / Leptomeningeal Solid Tumors					• Late 2018 / Early 2019 – Submit IND in B7-H3 Positive LM tumors
hu-Omburtamab	B7-H3	Systemic Solid Tumors (Adult) (Third-Line)					• 2019 – Submit IND
Naxitamab-BsAb	GD2xCD3	Refractory GD2-Positive Solid Tumors					• 1H 2018 – Submit IND
huCD33-BsAb	CD33xCD3	Hematological Cancers Expressing CD33					• 2019 – Submit IND

- (1) Pivotal registration study supportive of a BLA submission to the FDA, comprised of Study 12-230 measuring pharmacokinetic, toxicity and efficacy and an additional Phase 3 study, Study 201, to prove comparability between study sites using a cGMP commercial manufacturer and that has also been designed to satisfy the confirmatory study and post-marketing requirements by the FDA.
- (2) Initial study represents pediatric and young adult patients.
- (3) Represents the radioactive isotope of iodine used to radiolabel omburtamab.
- (4) Pivotal registration study supportive of a BLA submission to the FDA, comprised of Study 03-133 measuring pharmacokinetic, toxicity and efficacy and an additional Phase 3 study, Study 101, to prove comparability between study sites using a cGMP commercial manufacturer and that has also been designed to satisfy the confirmatory study and post-marketing requirements by the FDA.
- (5) Omburtamab-DTPA is a DTPA-conjugated omburtamab labeled with Lutetium-177 as chelator.

## Our Business Strategy

Our mission is to become the world leader in developing better and safer antibody-based pediatric oncology products addressing clear unmet medical needs and, as such, have a transformational impact on the lives of patients. We intend to advance and expand our product pipeline into certain adult cancer indications either independently or in collaboration with potential partners.

Key elements of our strategy to achieve this goal are:

- **Rapidly and concurrently advance our lead product candidates to regulatory approval.** We are currently in pivotal stage development for both of our lead product candidates, naxitamab for the treatment of pediatric R/R high-risk NB and <sup>131</sup>I-omburtamab for the treatment of pediatric CNS/LM from NB. We are advancing both of our lead product candidates through an expedited regulatory pathway and we expect that they will be eligible

for priority review. We expect to submit a BLA for each of our two lead product candidates in 2018, with a goal of receiving approval by the FDA in 2019. We plan to commercialize both of our lead product candidates in the United States as soon as possible after obtaining FDA approval, if such approval occurs.

- **Expand the indications and target patient populations for our existing product candidates.** Our goal is to maximize the potential of our existing product candidates in areas where there is a significant unmet medical need by exploring additional indications, as well as expanding the target population within existing indications. For example, we are developing naxitamab for the treatment of front-line NB and relapsed osteosarcoma and we intend to discuss our BLA strategy in these indications with the FDA after completing the BLA submission for naxitamab in pediatric R/R high-risk NB. We are also currently developing radiolabeled omburtamab for the treatment of pediatric patients with DIPG and DSRCT, both currently in Phase 1/2 clinical trials. After completing the BLA submission for <sup>131</sup>I-omburtamab for pediatric CNS/LM from NB, we intend to discuss with the FDA the protocol for the continuation and expansion of the ongoing DIPG and DSRCT clinical trials. We believe that we may qualify for a supplemental BLA, or sBLA, in each of these indications assuming positive pivotal data.
- **Independently commercialize our product candidates in indications and territories where we believe we can maximize their value.** We plan to independently commercialize our late-stage product candidates focusing on already-identified key treatment centers such as MSK, as well as educating doctors, patients and payors about our product candidates and their indications to drive acceptance and uptake. We believe that we will need to engage a small number of physician specialists for training regarding the appropriate administration and use of our product candidates. The sales call points for our current product candidates in the United States and the European Union are highly concentrated and generally addressable by a relatively small commercial organization, which we believe will allow us the flexibility to cost-effectively build our own commercial capability. Finally, in indications and in territories that are better served by the resources of larger biopharmaceutical companies we intend to form commercial and development collaborations.
- **Advance our novel BsAb product candidates that we believe may offer potential substantial benefits over existing bispecific constructs.** We are also advancing a promising pipeline of BsAbs that we believe have the potential to overcome limitations associated with existing BsAb constructs. Our first BsAb product candidate, naxitamab-BsAb, is a bivalent humanized anti-GD2 and anti-CD3 BsAb. We plan to submit an IND for this BsAb product candidate for the treatment of GD2-positive refractory solid tumors in the in the first half of 2018. We are also advancing our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33 and expect to file an IND in 2019. Further, we plan to utilize our access to the MULTI-TAG technology platform to create a diverse platform of dimerized BiTEs and are currently working with MSK on developing several MULTI-TAG candidates with a goal of selecting a candidate in 2018.
- **Leverage our relationships with leading academic and clinical institutions to develop additional product candidates.** We intend to continue to partner with leading centers, such as MSK, for cancer treatment worldwide, to identify and develop additional product candidates. We believe that our relationship with MSK, our access to several of their technologies and MSK's significant expertise in pediatric cancer care provides us with significant competitive advantages. For example, our Investigator-Sponsored Master Clinical Trial Agreement, or the MCTA, with MSK provides us with ready access to patients for clinical trial enrollment, which is a significant advantage in rare disease drug development where patients are often hard to locate and recruit. Our Sponsored Research Agreement, or

the SRA, with MSK, pursuant to which we agreed to provide research funding to MSK, grants us a first option to negotiate an exclusive license to MSK's rights in any new joint inventions discovered under the SRA. We plan to leverage our strong relationship with institutions such as MSK and their expertise and research capabilities to augment our own capabilities in order to identify new product candidates for the treatment of cancers where there is a significant unmet medical need and no effective therapy currently available.

## **Current Approaches to the Treatment of Cancer**

### ***Cancer Overview***

Cancer is a broad group of diseases in which cells divide and grow in an uncontrolled fashion, forming malignancies that can invade other parts of the body. Cancers can subsequently spread throughout the body by processes known as invasion and metastases. Cancer cells that arise in the lymphatic system and bone marrow, or BM, are referred to as hematological malignancies. Cancer cells that arise in other tissues or organs are referred to as solid tumors.

Cancer is a major public health problem in the United States and worldwide. The American Cancer Society, or ACS, estimated that approximately 40% of all men and women in the United States will be diagnosed with cancer during their lifetime (based on 2011-2013 data). According to the U.S. Centers for Disease Control, cancer is currently the second leading cause of death in the United States, and is expected to surpass heart disease as the leading cause of death in the next several years. Although progress has been made in the diagnosis and treatment of cancer, the ACS estimates that over 1.6 million new cancer cases will be diagnosed in the United States and over 600,000 people will have died from cancer in 2017. Thus, there remains a significant need for novel and improved treatment options for cancer patients.

Cancer treatment has traditionally included chemotherapy, radiation, hormone therapy, surgery or a combination of these approaches. While small molecule chemotherapy agents and cytotoxic agents have demonstrated efficacy in treating certain types of cancers, they can also cause toxicities that may lead to life-threatening consequences, lower quality of life or untimely termination of treatment. Furthermore, these treatments are only partially effective in solid tumors, in part because the maximal achievable doses are limited by systemic toxicity, which consequently hinders the prospects of long-term remission in patients. In the last 20 years, cancer research and treatment has shifted to more targeted therapies, such as monoclonal antibodies, and immuno-oncology, a new field of cancer therapy focused on enhancing antitumor immune responses.

Advances in understanding the immune system's role in treating cancer have established immunotherapy, or the practice of harnessing immune system functions to combat malignant cell growth, as an important treatment approach. Cancer immunotherapy began with treatments that nonspecifically activated the immune system and had limited efficacy and/or significant toxicity. In contrast, new immunotherapy treatments can activate specific, key immune cells, leading to improved targeting of cancer cells, efficacy, and safety.

Cancer therapies are sometimes characterized as front-line, second-line, or third-line, and the FDA often approves new therapies initially only for third-line use. When cancer is detected early enough, front-line therapy is sometimes adequate to effectively treat the cancer or prolong life. Whenever front-line therapy, usually chemotherapy, radiation therapy, surgery, or a combination of these, proves unsuccessful, second-line therapy may be administered. Second-line therapies often consist of more chemotherapy, surgery, antibody drugs, tumor-targeted therapies such as monoclonal antibodies and small molecule inhibitors, or a combination of these. Third-line therapies can include bone marrow transplantation, antibody and small molecule targeted therapies, more invasive forms of surgery, and new technologies.

## **Immune System and Introduction to Antibodies**

The immune system is often described as having two main branches—innate (non-specific) and adaptive (acquired) immunity. It defends against invading pathogens such as viruses, parasites, and bacteria, and provides surveillance against cancers. The innate immune system is the initial response to an infection, and the response is the same every time regardless of prior exposure to the infectious agent. The adaptive immune system includes B-cells, which secrete antibodies and T-cells, which can be either helper T-cells or cytotoxic T-cells.

An antibody, also known as an IgG, is a large, Y-shaped protein produced mainly by plasma cells in response to foreign substances, such as viruses or cancer cells. Antibodies circulating in the bloodstream function by binding to the target or antigen they are generated to fight. The binding process involves a lock-and-key mechanism in which the paratope region of the antibody, analogous to a lock, binds to one particular epitope of a specific antigen, analogous to a key. This allows the antibody to bind to a specific antigen with precision, thereby attacking only its intended target.

Different types of antibodies include: (i) *Monoclonal Antibodies*—laboratory-made antibodies typically derived from immune cells of mammals that have been immunized with a desired antigen and are all clones of a unique parent; (ii) *Humanized/Chimeric Antibodies*—antibodies with both mouse and human antibody proteins that are humanized (i.e., engineered to replace mouse components with more human components) to reduce the immune system response against antibodies identified as foreign (i.e., from a different species) in nature; (iii) *Naked Monoclonal Antibodies*—antibodies without any drug or radioactive material attached and which are the most common type of antibodies in treating cancer; (iv) *Antibody Drug Conjugates, or ADCs*—monoclonal antibodies that are joined to a chemotherapy drug, a radioactive particle or cancer cell killing agent, in which the monoclonal antibody is used as a homing device to deliver these substances directly to the cancer cell; and (v) *Bispecific antibodies* comprised of two different monoclonal antibody constructs, which allows the antibody to bind to two specific therapeutic targets at the same time, typically one target on the tumor cell and one target on an immune system cell.

Antibodies may function through multiple mechanisms simultaneously, including binding to cancer cells and flagging for B-cells and T-cells to more easily detect the target, or delivering radiation treatment by acting as a vehicle to transfer small radioactive particles directly to the cancer cells and to minimize the effect of radiation on normal cells. Other mechanisms include triggering cell-membrane destruction, preventing cell growth or blood vessel growth, blocking immune system inhibitors, directly attacking cancer cells and delivering chemotherapy or binding cancer cells and immune cells simultaneously.

Studies have shown that, as a drug class, antibodies have transformed oncology treatment and include some of the best-selling therapies on the biopharmaceutical market. Drugs derived from antibodies were the fastest growing subsegment of the global biopharmaceutical market in 2016 with \$81.9 billion in sales, representing approximately 42% of total biopharmaceutical sales and 10% of the global market for prescription drugs.

### **Our Product Candidates**

We have a broad and advanced product pipeline including two late-stage and clinically validated product candidates, naxitamab and omburtamab, which target tumors that express GD2 and B7-H3, respectively. Naxitamab and omburtamab are currently in pivotal stage development for pediatric R/R high-risk NB and pediatric CNS/LM from NB, respectively, both rare and life-threatening pediatric cancers for which no FDA approved products currently exist. We expect to submit a BLA for each of our two lead product candidates in 2018, with a goal of receiving approval by the FDA in 2019. We plan to commercialize both of our lead product candidates in the United States as soon as possible after obtaining FDA approval, if such approval occurs. Naxitamab and omburtamab are also in

mid-stage clinical development for additional cancers, and we have initiated clinical development for both product candidates in several other indications. Furthermore, we have two additional product candidates in pre-clinical development, omburtamab-DTPA and hu-omburtamab, a humanized version of omburtamab, each targeting indications with large adult cancer patient populations where there is a significant unmet medical need. We are also advancing a pipeline of BsAb product candidates through late pre-clinical development, including our naxitamab-BsAb product candidate for the treatment of GD2-positive refractory solid tumors and CD33-BsAb for the treatment of CD33-positive hematological cancers. We have exclusive worldwide commercial rights to all of our current product candidates.

### ***Naxitamab Overview***

Naxitamab is a humanized monoclonal antibody being evaluated for the treatment of R/R NB and other GD2-positive tumors, including osteosarcoma. Naxitamab targets GD2, which, based on our research, is expressed on almost all of NB cancer cells regardless of disease stage and in almost all osteosarcomas. Naxitamab is currently in pivotal stage development for patients with pediatric R/R high-risk NB and has received ODD and RPDD from the FDA for the treatment of NB in 2013 and 2017, respectively. The RPDD qualifies us for receipt of a PRV upon approval of naxitamab for treatment of NB, if such approval occurs. Naxitamab has been administered to more than 200 patients in several clinical trials conducted at MSK since 2011 and has been generally well tolerated, with no significant long-term toxicities observed to date. In Study 12-230, of the 23 patients with pediatric R/R high-risk NB, with evaluable tumors and who did not have PD at study entry, 13 patients, or 57%, achieved a clinical response.

In pediatric R/R high-risk NB, we believe that naxitamab has multiple potential advantages over other GD2 targeting antibody-based therapies. In particular, the modest toxicity it exhibits allows for doses 2.5 times greater than the other GD2 targeting antibody-based therapies. Naxitamab also has a significantly shorter infusion time (approximately 30 minutes compared to 10 to 20 hours for other GD2 targeting antibody-based therapies being used in front-line therapy, which we believe is important given the pain associated with the therapy) and the ability to be administered in an outpatient setting (compared to hospitalization stays of four days or longer for other GD2 targeting antibody-based therapies).

Based on our discussions with the FDA, profile of the non-PD pediatric R/R high-risk NB patients in Study 12-230 is representative of the intended patient population for our target indication. The corresponding ORRs will form the primary objective of our pivotal study (Study 201). Additionally, based on our discussions with the FDA, we believe that a 30% ORR (which is significantly different from a 20% ORR at a 95% confidence interval, or CI) with a minimum 12-week duration of response may qualify naxitamab for accelerated approval. We have proposed to the FDA that, pending comparability between the study population and the pharmacokinetics analysis in Study 12-230 and Study 201, the data from the two studies may be pooled for analysis for our planned BLA submission. In addition, naxitamab is currently being evaluated in a Phase 2 clinical study (Study 16-1643) in front-line NB and a Phase 2 clinical study (Study 15-096) in second-line relapsed osteosarcoma patients.

### ***GD2 Overview***

We believe that monoclonal antibodies such as naxitamab that target ganglioside GD2 are one of the most promising cancer immunotherapy approaches. Gangliosides, including GD2, GM2, GD3, NGcGM3 and OAcGD2, have been shown to be expressed at very high levels in tumor cells of several types of cancers.

As a potential target molecule for anti-tumor therapy, GD2 has certain advantages when compared to other tumor-associated gangliosides because it is highly expressed in tumor cells of several types of cancers and is not expressed at all, or expressed at very low levels, in normal cells. The



National Cancer Institute pilot program for the prioritization of the most important cancer antigens ranks GD2 as number 12 out of 75 potential targets for cancer therapy based on therapeutic function, immunogenicity, role of the antigen in oncogenicity, specificity, expression level and percent of antigen-positive cells, stem cell expression, number of patients with antigen-positive cancers, number of antigenic epitopes, and cellular location of antigen expression. GD2 ranks as number six when compared to antigens that are directly targetable on the cell surface. Antibodies directed against GD2 have been shown to effectively induce cell death through a combination of both apoptosis and tumor cell necrosis in GD2-positive tumors.

#### *GD2 Expression in Various Cancer Types*

Studies have shown that GD2 is highly expressed on neuroectoderm-derived tumors and sarcomas, including NB, retinoblastoma, melanoma, small cell lung cancer, brain tumors, osteosarcoma, rhabdomyosarcoma, Ewing's sarcoma in children and adolescents, as well as liposarcoma, fibrosarcoma, leiomyosarcoma and other soft-tissue sarcomas in adults. These cancers have a high mortality rate ranging from 20-80% depending on the tumor type.

We believe there is a large market opportunity for the treatment of solid tumors that express GD2. Based on our own research and our review of published research, we believe GD2 expression occurs in approximately 60-100% of tumor samples from various cancer types, and in substantially all NB and osteosarcoma tumor samples. We estimate that there were more than 200,000 new patients diagnosed with GD2-positive cancer in the United States in 2017. While our clinical development efforts for naxitamab are currently focused on rare pediatric cancers, we believe we have the potential to expand naxitamab's application beyond pediatric cancers to the treatment of adults with cancers that express GD2.

#### ***Naxitamab—Mechanism of Action***

Our pre-clinical studies have shown that naxitamab binds to GD2 molecules on tumor cells with high affinity and a slow off-rate, which indicates naxitamab's strong binding ability. In mice that have been transplanted with human NB tissue, naxitamab demonstrated dose-dependent inhibition of tumor growth (i.e., the effect of naxitamab varied with dosage) and generally increased survival. *In vitro* studies show that when naxitamab binds to tumor cells, it induces tumor cell death through antibody-dependent cell-mediated cytotoxicity and complement-dependent cytotoxicity. Naxitamab may also inhibit tumor cell migration through its inhibitory effect on GD2 molecules, which are involved in tumor cell adhesion and migration. *In vitro* studies also show that Granulocyte-Macrophage Colony Stimulating Factor, or GM-CSF, enhances the activity of naxitamab in a dose-dependent manner and is therefore generally combined with naxitamab in our clinical trials.

#### *Naxitamab for the Treatment of Pediatric Relapsed or Refractory High-Risk Neuroblastoma*

Naxitamab is currently in pivotal stage development (Study 201) for the treatment of pediatric R/R high-risk NB and received ODD and RPDD from the FDA for the treatment of NB in 2013 and 2017, respectively. The RPDD qualifies us for receipt of a PRV upon approval of naxitamab for treatment of NB by the FDA, if such approval occurs. In Study 12-230, we achieved an ORR of 57% in patients with pediatric R/R high-risk NB who had evaluable tumors and who did not have PD at study entry. Patients with these characteristics are the intended patient population for our first potential indication for treatment with naxitamab. Based on our discussions with the FDA, we believe that a 30% ORR (which is significantly different from a 20% ORR at a 95% CI) with a minimum 12-week duration of response may qualify for consideration of an expedited approval of naxitamab. We have proposed to the FDA that, pending comparability between the study population and the pharmacokinetics analysis in Study 12-230 and Study 201, the data from the two studies may be pooled for analysis. There would also be a post-marketing commitment to provide data on progression free

survival, or PFS, supporting the efficacy of the product. We believe naxitamab has multiple potential advantages over other GD2 targeting antibodies such as dosing, administration and safety.

In our studies to date, naxitamab has demonstrated relatively modest toxicity, which allows for 2.5 times greater dosing as compared to other GD2 targeting antibody-based therapies. This results in fewer doses per cycle and a significantly shorter infusion time (approximately 30 minutes versus 10 to 20 hours for dinutuximab). Notably, since severe pain is one of the most common side effects of treatment with GD2 targeting antibody-based therapies, we believe that the ability to reduce infusion time to approximately 30 minutes is very important for patients and may result in a significant reduction in demand for pain medication such as morphine. These factors allow naxitamab to be administered in an outpatient setting whereas other GD2 targeting antibody-based therapies require hospitalization which usually lasts for four days or more. In addition, unlike other GD2 targeting antibody-based therapies, we have not observed any life-threatening side effects with naxitamab to date.

#### *Overview of Neuroblastoma*

NB is a rare and almost exclusively a pediatric cancer that develops in the sympathetic nervous system, a network of nerves that carries messages from the brain throughout the body. It is the third most common childhood cancer, after leukemia and brain tumors, and is the most common solid extracranial tumor in children. NB is a life-threatening disease associated with poor long-term survival. It accounts for approximately seven percent of all childhood cancers and approximately 15% of pediatric cancer deaths. Nearly 90% of patients with NB are diagnosed by age five and NB is very rare in people over the age of 10 years. The average age of children when they are diagnosed with NB is one to two years.

The stage of NB, which describes how far the cancer has spread, is based on results of physical exams, imaging tests, and biopsies. The International Neuroblastoma Staging System stages the disease from Stage 1 to Stage 4. Other factors that also affect prognosis of NB include age and amplification of MYCN oncogene.

NB patients can also be placed into different risk groups from low, intermediate to high based on the stage and other prognostic factors. High-risk NB is defined as MYCN amplified Stage 2, 3, 4S and 4 in patients of any age and MYCN non-amplified Stage 4 in patients over 18 months of age.

Naxitamab is initially being evaluated for the treatment of pediatric R/R high-risk NB. There are approximately 700 children diagnosed with NB in the United States each year. We believe the European market is at least one and a half times the size of the U.S. market and that there are approximately 1,050 patients diagnosed with NB in Europe each year. We believe the current addressable market for naxitamab consists of approximately 675 new pediatric NB patients each year, representing approximately 40% of all pediatric patients diagnosed with NB in the United States and Europe, combined. Moreover, based on the protocol we have developed with MSK, between treatment and maintenance therapy, we believe that patients will receive on average 10 treatment cycles, or 30 doses, of naxitamab.

#### *Naxitamab for Pediatric Relapsed or Refractory High-Risk Neuroblastoma—Current Treatment Landscape and Associated Limitations*

Currently front-line treatment for pediatric NB patients usually occurs in three stages: induction, consolidation, and maintenance. During the induction phase, patients receive chemotherapy, radiotherapy and possibly surgery to eliminate as much tumor tissue and as many tumor cells as possible. Commonly used agents for induction treatment include cisplatin, etoposide, doxorubicin, cyclophosphamide, and vincristine. Following surgery and/or radiotherapy, most patients enter into consolidation therapy with the goal of eliminating any residual tumor usually with single dose myeloablative agents (e.g. carboplatin-etoposide-melphalan) with stem cell support or an autologous

stem cell transplant or repeated transplants with thiotepa-cyclophosphamide followed by cyclophosphamide, etoposide, and ranimustine. Many treatment centers also use immunotherapy as part of the consolidation stage of treatment.

Relapse is a frequent occurrence after consolidation. Although there are no approved therapies in the United States for R/R NB patients, treatments typically include chemotherapy, radiotherapy and other experimental therapies.

In 2015, the FDA and the European Medicines Agency, or the EMA, approved Unituxin (dinutuximab), a monoclonal GD2 targeting antibody developed by United Therapeutics Corporation, or United Therapeutics, and administered in combination with GM-CSF, interleukin-2, or IL-2, and isotretinoin, also known as 13-*cis*-retinoic acid, for the treatment of pediatric patients with high-risk NB who achieve at least a partial response, or PR, to prior front-line multiagent, multimodality therapy. The marketing authorization for Unituxin was voluntarily withdrawn by United Therapeutics in the European Union in 2017. Recently, the EMA approved Dinutuximab beta Apeiron (also known as dinutuximab beta, ch14.18/CHO, Isqette), a monoclonal GD2 targeting antibody, for the treatment of high-risk NB in patients aged 12 months and older, who have had some improvement with previous treatments or patients whose NB has not improved with other cancer treatments or has relapsed.

*Naxitamab for Pediatric Relapsed or Refractory High-Risk Neuroblastoma—Clinical Development Program*

An earlier murine version of naxitamab was studied in 17 clinical trials at MSK with a total of more than 800 patients over 25 years. Naxitamab has been studied in several clinical trials for the treatment of pediatric R/R NB and other diseases, of which Study 201, Study 12-230, Study 11-009, Study 15-096 and Study 16-1643 are currently ongoing. We expect to receive topline data from our ongoing pivotal trial (Study 201) in pediatric R/R high-risk NB and submit the BLA in the second half of 2018.

Based on our discussion with the FDA, ORR will form the primary objective for our pivotal Study 201. We have proposed to the FDA that, pending comparability analysis between study population and pharmacokinetic analysis in Study 12-230 and Study 201, the data from the two studies may be pooled to form the primary basis of our BLA. Based on our discussions with the FDA, we believe that a 30% ORR (which is significantly different from a 20% ORR at a 95% confidence interval) with a minimum 12 week duration of response may qualify for accelerated approval. Thirty-seven patients are expected to be included in Study 201. We expect that the safety portion of our planned BLA submission will be comprised of more than 200 patients treated with naxitamab across multiple indications.

*Study 12-230: Phase I/II Study of Combination Therapy of Antibody Naxitamab with Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) in Patients with Relapsed/Refractory High-Risk Neuroblastoma*

**Phase 1 Portion of Study 12-230**

*Primary Objective*

- To establish the maximum tolerated dosage, or MTD, of naxitamab when combined with GM-CSF.

*Secondary Objectives*

- To study the pharmacokinetics of naxitamab when combined with GM-CSF.
- To assess activity of naxitamab plus GM-CSF against NB.

- To quantitate pain during naxitamab and GM-CSF treatment.
- To study markers of granulocyte-mediated cytotoxicity and NK-mediated cytotoxicity, anti-naxitamab immunity, and anti-tumor immunity before and after treatment with naxitamab/GM-CSF.
- To quantitate the response of NB in BM by quantitative reverse-transcription-polymerase chain reaction, or RT-PCR.

Patient Population

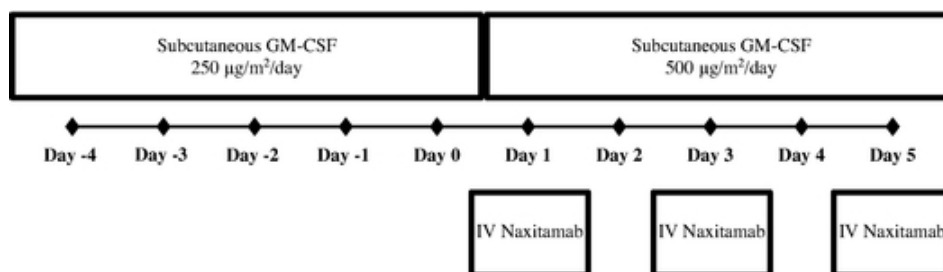
In addition to satisfying certain other criteria, patients must be over one year of age and must have been diagnosed with NB as defined by a) histopathology, or b) BM metastases or Meta-iodobenzylguanidine, or MIBG, avid lesion(s) plus high urine catecholamine levels.

Patients must have R/R high-risk NB (including MYCN-amplified Stage 2, 3, 4, or 4S of any age and MYCN-non amplified Stage 4 in patients over 18 months of age) resistant to standard therapy. Standard therapy for these types of patients includes intensive induction chemotherapy, followed by a variety of consolidation or salvage therapies, depending on response.

Patients will be mainly children and adolescents.

Treatment Protocol

The Phase 1 portion of Study 12-230 assessed dose escalation of intravenous, or IV, naxitamab (days one, three, five) in the presence of subcutaneous GM-CSF (days minus four through five). These three doses of naxitamab and 10 days of GM-CSF constituted a single treatment cycle. Patients were eligible for up to five cycles in the initial part of this treatment. The diagram below depicts the treatment schedule per cycle in Study 12-230:



Results of Phase 1 Portion of Study 12-230

A total of 57 patients were enrolled in the Phase 1 portion of Study 12-230 between December 2012 and May 2016. A summary of patient characteristics is provided in the table below.

**Study 12-230 patient characteristics (Phase 1)**

Measure	Value
Years from diagnosis	0.6 - 9.0 (median 3.1)
Age at study entry (years)	2.4 - 31.3 (median 6.8)
Prior anti-GD2 immunotherapy	47/57 (82%)
Autologous stem-cell transplantation	24/57 (42%)
<sup>131</sup> I-MIBG therapy	17/57 (30%)

All 57 patients were heavily treated prior to entering the study as indicated by the high number of patients previously receiving <sup>131</sup>I-MIBG (n=17) and anti-GD2 mAbs (n=47).

## Safety Results

MTD was not reached. The maximum dose used was 9.6 mg/kg per cycle. This dose was more than 2.5 times greater than the doses that can be given when using the earlier murine version of naxitamab or dinutuximab, and manageable acute side effects allowed treatment to occur in an outpatient setting. DLTs occurred in four of 57 patients. Two patients developed hypersensitivity, one patient experienced hypertension and one patient experienced renal failure.

## Pharmacokinetic Results

The protocol requires patients to be administered naxitamab at dose levels from 0.3 to 3.6 mg/kg per dose on days one, three, and five of a cycle (0.9 to 10.8 mg/kg per cycle).

## Human Anti-human Antibody (HAHA) Results

Of the 57 patients, four patients received one to two cycles of naxitamab and the earlier murine version of naxitamab, and of those four patients, one patient, developed human anti-human antibody, or HAHA, response.

## Efficacy Results

Evidence of anti-NB activity was observed at all dose levels; however, a dose-response relationship was not possible due to intra-patient dose escalation after two cycles as permitted by the protocol.

After excluding two patients with early DLT, 55 of 57 patients were included in the overall analysis of efficacy. Of these 55 patients at study entry, 25 patients had no evidence of disease, or NED, and 30 patients had evaluable disease. Of the 30 patients with evaluable disease, seven patients had PD at study entry.

Of the remaining 23 non-PD patients with primary or secondary refractory disease, 13 patients achieved either a complete response (also known as complete remission), or CR, or a PR, which resulted in an ORR of 57% (13/23). Further, one patient had SD, another six patients had PD and two patients were only available for short term follow-up (long term data not available).

As shown in the table below, eight of 11 primary refractory patients achieved an ORR of approximately 73%, and five of 12 secondary refractory patients achieved an ORR of approximately 42%.

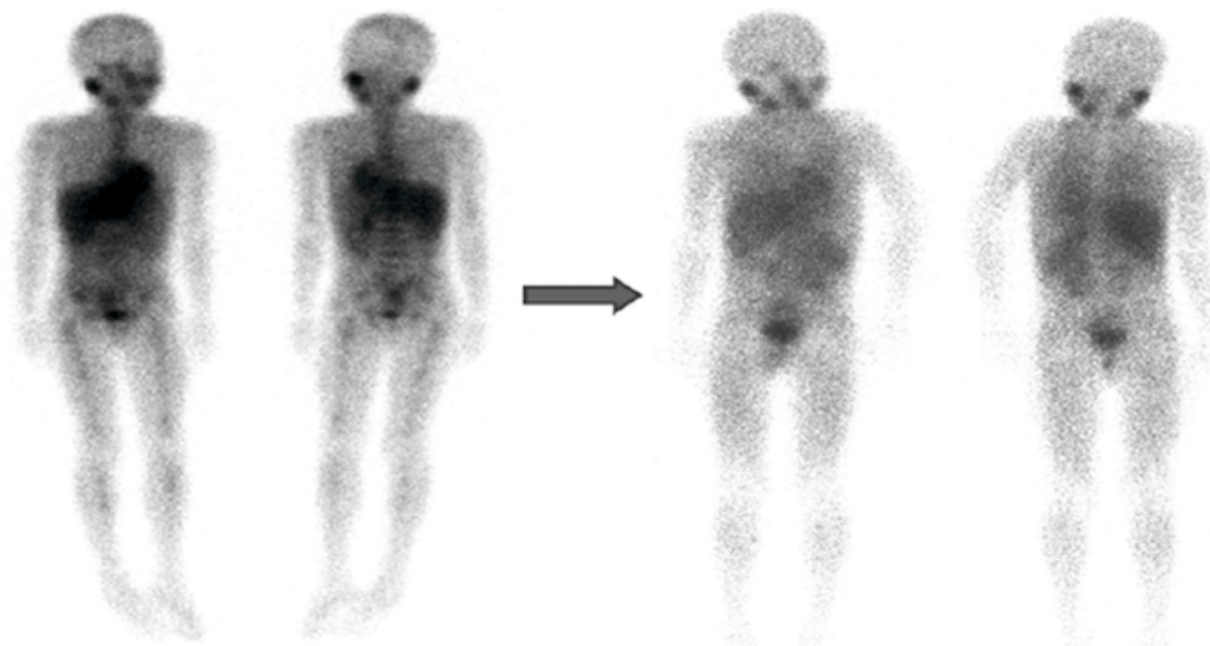
### **Study 12-230 efficacy results among non-PD patients (Phase 1)**

<b>Patient group</b>	<b>CR/PR</b>	<b>SD</b>	<b>PD</b>	<b>Short f/u</b>
Primary refractory (n = 11)	8 (72.7%)	1 (9.1%)	1 (9.1%)	1 (9.1%)
Secondary refractory (n = 12)*	5 (41.7%)	0 (0%)	5 (41.7%)	1 (8.3%)
<b>All patients with non-progressive evaluable disease (n = 23)</b>	<b>13 (56.5%)</b>	<b>1 (4.3%)</b>	<b>6 (26.1%)</b>	<b>2 (8.7%)</b>

CR = complete response; PR = partial response; SD = stable disease; PD = progressive disease; Short f/u = Short-Term follow-up

\* One of the 12 patients developed anaphylaxis and was removed from the study at seven months.

The scan on the left below shows multiple  $^{123}\text{I}$ -MIBG hot spots (NB lesions) localized to the bone and BM. In the scan on the right below, taken after naxitamab and GM-CSF treatment, nearly all the metastatic lesions have disappeared. Although not every patient will experience similar results, we believe these scans are indicative of a patient that has responded favorably to naxitamab and GM-CSF treatment.



Among the 25 patients with NED, it was not possible to classify response by International Neuroblastoma Response Criteria, or INRC criteria, including with  $^{123}\text{I}$ -MIBG. These patients, who had one to five prior relapses and therefore had a poor prognosis, showed an encouraging two-year EFS of 24%.

**Treatment in Study 12-230 with naxitamab in patients previously exposed to other anti-GD2 antibodies (dinutuximab or earlier murine version of naxitamab)**

A large proportion of the patients (n=47) had previously been treated with anti-GD2 mAbs. We have also demonstrated that naxitamab has efficacy when used following front-line treatment with dinutuximab. A survival analysis was completed in all 16 patients with prior exposure to dinutuximab.

**Phase 2 Portion of Study 12-230**

The Study 12-230 protocol was amended in May 2016 to include an expansion Phase 2 portion. As of October 2017, 114 patients had been enrolled, inclusive of the initial 57 patients from the Phase 1 portion of the study.

The expansion Phase 2 single-arm portion of Study 12-230 was designed to assess the anti-NB activity of naxitamab and GM-CSF in patients who presented with lesions that could be objectively measured and/or monitored by  $^{123}\text{I}$ -MIBG scans and who were deemed to have measurable disease and be eligible for response classification by the INRC classification incorporating  $^{123}\text{I}$ -MIBG scans. These patients were classified as having evaluable disease and consisted of patients that were primary refractory patients or secondary refractory patients. Another group of patients included those with NED but with a high risk of relapse.

## Patient Population

In addition to satisfying certain other criteria, patients must be over one year of age and will be mainly children and adolescents.

### *Primary Objectives*

- In Group 1: To assess the activity of naxitamab and GM-CSF in patients who have primary refractory disease in BM by measuring response and by calculating PFS.
- In Group 2: To assess the impact of naxitamab and GM-CSF on PFS in patients in greater than or equal to second CR/VGPR, but at high-risk of another relapse.
- In Group 3: To assess the activity of naxitamab and GM-CSF in patients who have secondary refractory disease in BM by measuring response and by calculating PFS.

### *Secondary Objectives*

- To apply real-time quantitative RT-PCR to test the hypothesis that the minimal residual disease, or MRD, findings in BM after the first two cycles of naxitamab and GM-CSF have significant prognostic impact on outcome.

Currently, no published safety or efficacy data is available from the Phase 2 portion of the study.

### *Study 201: A Phase 3 Trial of Antibody Naxitamab and Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) in High-Risk Neuroblastoma Patients with Primary or Secondary Refractory Osteomedullary Disease*

Study 201 is a single-arm multi-center pivotal study using current Good Manufacturing Practices, or cGMP, manufactured naxitamab, which commenced recruitment in the first quarter of 2018. We expect to enroll a total of 37 patients.

## Patient population

In addition to satisfying certain other criteria, patients must have high-risk NB with primary or secondary refractory osteomedullary disease. Primary refractory disease is defined as no prior relapse but incomplete response to treatment in BM as documented by histology and/or <sup>123</sup>I-MIBG scan. Secondary refractory disease is defined as prior relapse and incomplete response to salvage therapy in BM as documented by histology and/or <sup>123</sup>I-MIBG scan. Patients must be older than one year of age.

## Treatment Protocol

Study 201 will follow the same treatment protocol as previously described for Study 12-230 above.

### *Primary Objective*

- To evaluate the efficacy of IV naxitamab and GM-CSF.

### *Secondary Objectives*

- To evaluate the safety of IV naxitamab and GM-CSF.
- To evaluate the duration of response from the start of naxitamab and GM-CSF. Duration of response is defined as the length of time from patient response to PD.

- To evaluate PFS of naxitamab and GM-CSF.
- To evaluate median OS at two years following naxitamab and GM-CSF.
- To evaluate the pharmacokinetics of naxitamab and investigate the formation of HAHA.

*Study 16-1643: Naxitamab/GM-CSF Immunotherapy Plus Isotretinoin for Consolidation of First Remission of Patients with High-Risk Neuroblastoma: A Phase II Study*

Study 16-1643 is a Phase 2 single-arm clinical trial where patients with high-risk NB in first CR/VGPR undergo consolidation with naxitamab and GM-CSF for five cycles and isotretinoin for six cycles. The primary objective of the study is to determine relapse-free survival following treatment with naxitamab combined with GM-CSF and isotretinoin. As of October 2017, 12 patients had been enrolled in the study.

Patient population

In addition to satisfying certain other criteria, patients must have diagnosis of NB as defined by a) histopathology, or b) BM metastases or MIBG-avid lesion(s) plus high urine catecholamine levels. Patients must have high-risk NB (MYCN-amplified Stage 2, 3, 4, and 4S of any age and MYCN-nonamplified Stage 4 in patients above 18 months of age). Patients must be in first CR/VGPR.

Patients will mainly be children and adolescents.

Treatment protocol

The dosing and regimen for naxitamab and GM-CSF is similar to the protocol in Study 12-230. Naxitamab and GM-CSF is given for five cycles and isotretinoin for six cycles. In addition to naxitamab and GM-CSF, isotretinoin, which has been shown to decrease the risk of relapse in patients treated in CR, is administered at 160mg/m<sup>2</sup>/d, divided into two doses, for 14 days. This treatment can be repeated after a minimum rest period of 14 days, for a total of six cycles starting after two cycles of naxitamab and GM-CSF unless HAHA develops and precludes timely administration of cycle 2 of naxitamab and GM-CSF. The interval between end of a treatment cycle of naxitamab and GM-CSF and start of next treatment cycle is two to four weeks through cycle 4, then the interval is up to six to eight weeks until cycle 5.

*Primary Objective*

- To determine two years relapse-free survival.

*Secondary Objective*

- To determine MRD by using BM specimens.

*Study 11-009: Phase I Study of Naxitamab Monoclonal Antibody in Patients with High-Risk Neuroblastoma and GD2-Positive Tumors*

Study 11-009 is a Phase 1 clinical dose escalation study with IV naxitamab given as monotherapy in patients with high-risk NB or other GD2-positive tumors. We intend to use the safety data from this study, when available, to support our planned BLA submission for naxitamab in pediatric R/R high-risk NB. As of October 2017, 68 patients had been enrolled in the study, and we expect to enroll a total of 74 patients. The primary objective of the study is to establish the MTD of naxitamab. The secondary objectives are to study the pharmacokinetics, to assess activity of naxitamab against NB and other GD2-positive tumors, and to quantitate pain during naxitamab treatment. As of



October 2017, a MTD had not been reached in the study. Two patients experienced reversible DLT of elevated liver transaminases.

## **Naxitamab for the Treatment of Relapsed Osteosarcoma**

Naxitamab is currently being evaluated in an ongoing Phase 2 clinical study (Study 15-096) for the treatment of patients with relapsed osteosarcoma that have been rendered surgically free of evident disease. As of October 2017, 14 patients had been enrolled and we expect to enroll a total of 39 patients. The trial is designed to distinguish between 12-month EFS of 30% versus 50%.

### *Overview of Osteosarcoma*

Osteosarcoma is the most commonly diagnosed primary malignancy of bone, particularly among children and adolescents. It is relatively rare and represents less than one percent of all cancers diagnosed in the United States. According to the ACS, most osteosarcomas occur in children and adolescents between the ages of 10 and 30. In young patients, it most often arises in the metaphyses of long bones, such as the distal femur, proximal tibia, and proximal humerus.

Each year, approximately 1,000 new patients are diagnosed with osteosarcoma in the United States. Assuming similar prevalence as in the United States, we estimate approximately 1,500 patients diagnosed with osteosarcoma per year in Europe. If approved, we would expect to treat approximately 300 patients per year in the United States and Europe, combined.

### *Naxitamab for Relapsed Osteosarcoma—Current Treatment Landscape and Associated Limitations*

Current treatment options for front-line and relapsed osteosarcoma consist of surgery, chemotherapy, radiotherapy, or a combination of the three. Multimodality treatment is increasingly recognized as an important approach for increasing a patient's chance of prolonged survival. Approximately 50% to 70% of patients treated with aggressive surgical resection and systemic therapy (combination methotrexate, doxorubicin, and cisplatin chemotherapy) achieve long-term EFS if they have localized disease at diagnosis. However, as discussed below, the prognosis for patients with metastatic disease at diagnosis or those with relapsed disease is very poor. Over the past three decades, several attempts at improving the prognosis for these patients have achieved little success. Strategies that incorporated dose-intensification of existing agents or addition of other conventional chemotherapeutic agents as well as biological agents, have not achieved long-term benefit in patients with relapsed osteosarcoma. We believe that at present, there are no novel compounds that have demonstrated activity in relapsed osteosarcoma and few therapeutic options exist for patients with relapsed disease.

The poor prognosis in relapsed osteosarcoma has been confirmed in several reports. A study from the Cooperative Osteosarcoma Study Group reported that while only one of 205 patients with recurrence survived past five years without surgical resection, the five-year OS and EFS rates were 32% and 18% for second recurrence, 26% and 0% for third recurrence, 28% and 13% for fourth recurrence, and 53% and 0% for fifth recurrence, respectively, in which a renewed surgical remission was achieved.

### *Naxitamab for Relapsed Osteosarcoma—Clinical Development Program*

Currently, naxitamab is being evaluated in an ongoing Phase 2 clinical trial (Study 15-096) for the treatment of relapsed osteosarcoma. This Phase 2 clinical trial is designed to assess the efficacy of naxitamab when combined with GM-CSF in patients with relapsed osteosarcoma who have been rendered surgically free of evident disease. The study commenced in July 2015, and as of October 2017, 14 patients had been enrolled. We expect to recruit a total of 39 patients. This trial is designed to distinguish between a 12-month EFS of 30% versus 50%.

*Study 15-096: A Phase II Study of Monoclonal Antibody Naxitamab with Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) in the Treatment of Recurrent Osteosarcoma*

Study 15-096 is a Phase 2 clinical trial to assess the efficacy of the humanized anti-GD2 antibody, naxitamab, when combined with GM-CSF, in patients with recurrent osteosarcoma who have been rendered surgically free of evident disease.

Patient Population

In addition to satisfying certain other criteria, patients must be older than one year and up to 40 years of age. To enroll, patients must have a diagnosis of relapsed osteosarcoma. Patients must also be in or beyond their second CR.

Treatment Protocol

Each cycle of therapy is 10 days. The treatment protocol defined one cycle of treatment with IV naxitamab at a dose of 2.4 mg/kg/dose for three days (days one, three, and five) in the presence of subcutaneous GM-CSF (administered on day minus four before dose one of naxitamab). These three doses of naxitamab with GM-CSF administered subcutaneously before dose one of naxitamab constitute a treatment cycle. Cycles can be repeated at two to four week intervals between first days of naxitamab, through five cycles. A maximum of five cycles were administered on protocol. No simultaneous anti-cancer therapy was permitted while on study.

The primary objective of the study is to evaluate EFS at 12 months and secondary objectives are to evaluate time to recurrence, OS and toxicity associated with naxitamab and GM-CSF.

**Omburtamab Overview**

Omburtamab is a novel murine monoclonal antibody currently designed for compartmental immunotherapy, for example in the CNS. Omburtamab targets B7-H3, an immune checkpoint molecule that is widely expressed in tumor cells of several types of cancers. We have radiolabeled omburtamab with either Iodine-131 (<sup>131</sup>I-omburtamab) or Iodine-124 (<sup>124</sup>I-omburtamab). <sup>131</sup>I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB, and was granted ODD, RPDD and BTD in this indication in 2016-2017. The RPDD qualifies us for receipt of a PRV upon approval of omburtamab for treatment of NB, if such approval occurs. An analysis of 93 treated patients treated through August 2017 demonstrated median OS of 47 months (including a five-year median OS of approximately 43%), as compared to historical median OS of approximately six months. Omburtamab has also been generally well tolerated in over 200 patients treated over 14 years for multiple indications, with no significant long-term toxicities. We expect to submit the BLA for <sup>131</sup>I-omburtamab for treatment of patients with R/R NB who have CNS or LM in the second half of 2018. In addition, radiolabeled omburtamab is in Phase 1/2 clinical development for two additional rare pediatric cancers, DSRCT and DIPG, with clinical results expected for both in the first half of 2018. We believe that we are well positioned to submit sBLAs in each of these two indications, assuming positive results in these Phase 1/2 clinical trials. Further, we believe that omburtamab has the potential to address several other tumors in children and adults that express B7-H3 such as prostate, ovarian, breast, colon, renal, non-small cell lung, pancreatic, head and neck cancers, as well as melanoma, glioblastoma, and NB and other small round blue cell tumors of childhood.

**B7-H3 Overview**

B7-H3 is a member of the B7 family of immune-regulatory ligands. The family includes B7-1, B7-2, PD-L1, PD-L2, B7-H3, B7-H4, B7-H6 and their ligands on T-cells PD-1, CD28, CTLA-4 and ICOS. B7-H3 is highly expressed on many solid cancers and displays high tumor-versus-normal tissue binding differential. In mice, studies have shown that members of the B7 family have the capability to

regulate the immune system through both stimulatory and inhibitory signals. Inhibition of certain members of the B7 family has been shown to have significant anti-tumor effects in several solid tumor types. As such, we believe that B7-H3 is a promising target for designing targeted therapeutics with a range of modalities.

#### *B7-H3 Expression in Various Cancer Types*

Studies have shown that B7-H3 is highly expressed on a variety of solid cancer tumors, including prostate, ovarian, breast, colon, renal, non-small cell lung, pancreatic, head and neck cancers, as well as melanoma, glioblastoma, and NB and other small round blue cell tumors of childhood. In addition, a high degree of B7-H3 expression on solid tumors has been correlated with greater disease severity, poor outcomes and worse median OS in a number of these cancer types.

We believe there is a large market opportunity for the treatment of solid tumors that express B7-H3, with hundreds of thousands of new cases estimated in the United States in 2017. Based on our review of published research, we believe that B7-H3 expression occurs in a range of 70% to 100% of tumor samples for various cancer types, which makes B7-H3 a promising immunotherapy target. Our literature review also revealed that B7-H3 expression on the systemic tumor is replicated in the metastasized tumor. While our clinical development efforts for omburtamab are currently focused on rare pediatric cancers, we believe we have the potential to expand omburtamab's application to both the treatment of CNS/LM from solid tumors that express B7-H3 and the underlying solid systemic tumor. As part of Study 03-133, we have also treated a small number of adult patients with solid tumors that have metastasized to the CNS/LM compartment with <sup>131</sup>I-omburtamab and preliminary indications show that it has been generally well tolerated and promising results.

#### **<sup>131</sup>I-omburtamab and <sup>124</sup>I-omburtamab—Mechanism of Action**

<sup>131</sup>I-omburtamab and <sup>124</sup>I-omburtamab are monoclonal antibodies that are radiolabeled with either Iodine-131 or Iodine-124, respectively, and both target B7-H3. Upon administration, radiolabeled omburtamab binds selectively to B7-H3 ligand that is expressed on the tumor cell surface. Both Iodine-131 and Iodine-124 emit beta radiation, resulting in deoxyribonucleic acid, or DNA, damage and tumor cell death. Beta radiation from both iodine isotopes penetrates 1-3 mm, affecting not only the antibody bound cell but also the neighboring tumor cells. Iodine-131 has a half-life of eight days while Iodine-124 has a half-life of four days. In contrast to Iodine-131, which emits electrons, Iodine-124 is a positron-emitting iodine isotope, enabling measurement of iodine uptake using positron emission tomography, or PET scans. This is important when using radiotherapy in a critical organ such as pons, where overdosing may have serious consequences. Radiolabeling of omburtamab with either Iodine-124 or Iodine-131 takes place at qualified radiopharmacies according to a well-established procedure.

#### **<sup>131</sup>I-Omburtamab for the Treatment of Pediatric Central Nervous System/Leptomeningeal Metastases from Neuroblastoma**

<sup>131</sup>I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB, and has been granted ODD, RPDD and BTM by the FDA in this indication in 2016-2017. The RPDD qualifies us for receipt of a PRV upon approval of omburtamab for treatment of NB, if such approval occurs. At our meeting with the FDA in June 2017, we proposed to the FDA that data from Study 03-133 may be pooled with data from Study 101 and utilized for our planned BLA submission. As of August 2017, 93 patients with pediatric CNS/LM from NB had been treated with <sup>131</sup>I-omburtamab in Study 03-133. An analysis of these 93 patients demonstrated a median OS of 47 months (including an estimated five-year OS of approximately 43%), as compared to historical median OS of approximately six months. Omburtamab has also been generally well tolerated in over 200 patients treated at MSK over 14 years for multiple indications, with no significant long-term toxicities. <sup>131</sup>I-omburtamab can be administered as a push injection in an outpatient setting. We expect

to submit a BLA for each of our two lead product candidates in 2018, with a goal of receiving approval by the FDA in 2019. We plan to commercialize both of our lead product candidates in the United States as soon as possible after obtaining FDA approval, if such approval occurs.

#### *Overview of Central Nervous System/Leptomeningeal Metastases from Neuroblastoma*

CNS/LM is a rare and usually fatal complication of NB in which the disease spreads to the membranes, or meninges, surrounding the brain and spinal cord in the CNS. In CNS/LM from NB, the CNS has emerged as a sanctuary site for NB tumor cells leading to relapse with an incidence of CNS/LM from NB of approximately 6% to 10%. It is expected that the incidence of CNS/LM from NB disease will increase concurrently with better treatment options for systemic NB, as more patients achieve longer systemic remissions allowing for more CNS relapses. Relapsed metastatic NB is difficult to treat particularly in patients with R/R NB who have CNS or LM. The median OS after detection of the CNS/LM from NB is approximately six months even with early detection and intervention.

Omburtamab is currently being evaluated for the treatment of CNS/LM from NB. There are approximately 700 children diagnosed with NB in the United States each year. Of these, approximately 50-60% are high-risk, and of those at high-risk, we believe approximately 20% will suffer from CNS/LM from NB. A published study analyzing frozen sections from tumors with histologically confirmed diagnosis of NB using immunohistochemistry showed 87 out of 90 sections (or approximately 97%) were B7-H3 positive. We believe the European market is at least one and a half times the size of the U.S. market and that there are approximately 1,050 patients diagnosed with NB in Europe each year. We believe the current addressable market for our product candidate, omburtamab, consists of approximately 200 new patients each year with CNS/LM from NB, representing approximately 11% of all new pediatric patients diagnosed with CNS/LM from NB in the United States and Europe, combined.

#### *<sup>131</sup>I-omburtamab for Pediatric Central Nervous System/Leptomeningeal Metastases from Neuroblastoma—Current Treatment Landscape and Associated Limitations*

There are currently no approved products for patients with R/R NB who have CNS or LM. A variety of treatments are used alone and in combination with other treatments. It is widely accepted that no effective treatment regimens for CNS/LM from NB are available, and the goals of treatment are generally palliative. For recurrence in the CNS, the therapeutic approach consists primarily of surgery, radiation therapy and/or chemotherapy. These treatments have had very limited success, with median OS of approximately six months. The current standard of care treatment paradigm typically involves the following:

- Surgery—for debulking the tumor prior to irradiation and chemotherapy and to reduce edema and hemorrhage;
- Radiation—focal, craniospinal or whole brain irradiation used for symptom alleviation, cerebrospinal fluid, or CSF, flow correction or for debulking to facilitate chemotherapy; and/or
- Chemotherapy—standard combinations of chemotherapy such as irinotecan and temozolomide.

The uniformly poor outcomes associated with these different regimens highlight the significant unmet medical need for treatment of CNS/LM from NB:

1. Our recent review of published research representing 83 patients treated between 1979 and 2013 showed a median OS of 5.6 months (95% CI of three to eight months) for patients with R/R NB who have CNS or LM. We also performed a restricted analysis after removing patients who died before receiving therapy for the CNS/LM from NB

disease and only received palliative treatment, or who presented with rapidly progressing systemic disease. The restricted analysis comprised of 58 patients with a median OS of 8.7 months (95% CI of 5.8 to 11 months) after diagnosis of CNS/LM from NB. There were only three cases of survival beyond three years.

2. Data from 85 patients sourced from The Central German Childhood Cancer Registry, or CGCCR, showed a median OS of 4.7 months. The data was extracted from patients diagnosed between 1990 and 2010. It is estimated that more than 90% of all German childhood cancer patients are registered in this database.
3. Finally, our review of data from 19 patients treated at MSK prior to when  $^{131}\text{I}$ -omburtamab was first introduced in 2004, demonstrated a median OS of 5.5 months.

*$^{131}\text{I}$ -omburtamab for Pediatric Central Nervous System/Leptomeningeal Metastases from Neuroblastoma—Clinical Development Program*

Currently,  $^{131}\text{I}$ -omburtamab is in pivotal stage development for the treatment of pediatric CNS/LM from NB as a monotherapy after patients have completed standard of care treatment. At our meeting with the FDA in June 2017, we proposed to the FDA that data from Study 03-133 may be pooled with data from Study 101 and utilized for our planned BLA submission. As of August 2017, 93 patients with pediatric CNS/LM from NB had been treated with  $^{131}\text{I}$ -omburtamab in Study 03-133. In addition to the pivotal efficacy data currently available for these 93 patients, we also have safety data showing no significant long-term toxicities for more than 200 patients from multiple clinical trials including patients with other cancer types such as DIPG and DSRCT. We are planning to treat an additional 18 patients in a multi-center Phase 3 trial (Study 101) for the purposes of pharmacokinetic and dosimetry comparability between study sites using  $^{131}\text{I}$ -omburtamab from our cGMP commercial manufacturer, versus drug product previously produced by MSK. Study 101 has also been designed to satisfy the confirmatory study and post-marketing requirement by the FDA, and, as a result, we will continue to recruit 14 more patients in addition to the initial 18 patients required for the BLA submission. We expect to submit the BLA for  $^{131}\text{I}$ -omburtamab for treatment of patients with CNS/LM from NB in the second half of 2018.

*Study 03-133: Phase I Study of Intrathecal Radioimmunotherapy using  $^{131}\text{I}$ -omburtamab for Central Nervous System/Leptomeningeal Neoplasms*

The trial was originally designed as a Phase 1 clinical dose escalation study followed by cohort expansion at the recommended dose. To determine the MTD, patients received up to 70 mCi  $^{131}\text{I}$ -omburtamab as outpatients. Although not DLT, myelosuppression was observed in patients who had received craniospinal radiation and  $^{131}\text{I}$ -omburtamab at dose levels of 60 and 70 mCi, as a result a 50 mCi dose was chosen for subsequent enrollment. Once the therapeutic dose of 50 mCi was established, an extension phase was implemented by a protocol amendment. At our meeting with the FDA in June 2017, we proposed to the FDA that data from Study 03-133 may be pooled with data from Study 101 and utilized for our planned BLA. As of August 2017, 93 patients with pediatric CNS/LM from NB had been treated with  $^{131}\text{I}$ -omburtamab in Study 03-133. Of these 93 patients, 81 had been treated with 50 mCi  $^{131}\text{I}$ -omburtamab. We expect that the safety portion of the BLA will be comprised of data from more than 200 patients treated with  $^{131}\text{I}$ -omburtamab or  $^{124}\text{I}$ -omburtamab across multiple indications. Study 03-133 has been held open for recruitment even after the August 2017 data cutoff date so that we may be able to continue to offer this promising treatment to patients.

## Patient Population

In addition to satisfying certain other criteria, patients must have a histologically confirmed diagnosis of a malignancy known to be reactive to omburtamab, a B7-H3 binding antibody. Furthermore, patients must have CNS/LM from NB disease which is refractory to conventional therapies or for which no conventional therapy exists, or a relapsed brain tumor with a predilection for LM dissemination (primitive neuroectodermal tumor, rhabdoid tumor, medulloblastoma).

Before enrollment in Study 03-133, most patients underwent biopsy or debulking surgery to remove brain metastases as much as possible, followed by radiation therapy and chemotherapy. A majority of the patients were also treated with an anti-GD2 immunotherapy such as naxitamab to control systemic disease after completing the  $^{131}\text{I}$ -omburtamab treatment under Study 03-133. All patients had an intraventricular device implanted before enrollment in the study.

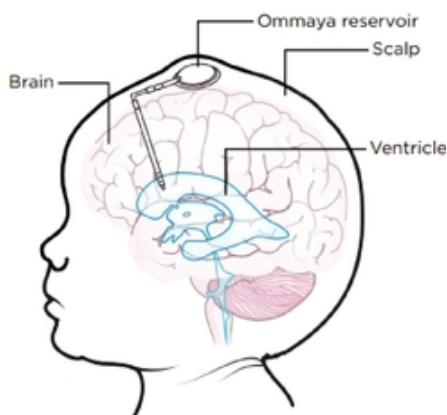
Approximately 80% of all CNS/LM from NB patients presenting at MSK since the initiation of the study were included in the study and the remaining patients were primarily excluded due to the fact that they had already received maximum dose of previous radiotherapy to CNS, or had progressive systemic disease.

## Treatment Protocol

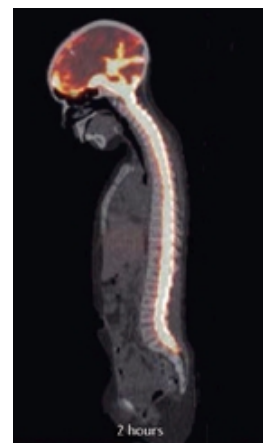
Patients are treated with up to two cycles (consisting of two treatment and dosimetry doses) of  $^{131}\text{I}$ -omburtamab administered through intrathecal infusion via an Ommaya reservoir by which the drug is distributed at the intrathecal space to the entire CSF, (as shown in the figure on the left below). A treatment cycle with  $^{131}\text{I}$ -omburtamab under Study 03-133 proceeds as follows:

- Week 1:  $^{131}\text{I}$ -omburtamab (dosimetry dose: 2-mCi imaging test dose);
- Week 2:  $^{131}\text{I}$ -omburtamab (treatment dose: 30-50 mCi depending on age);
- Weeks 3 and 4: observation period; and
- Week 5: post-treatment evaluation comprised of magnetic resonance imaging, or MRI, of the head and spine, CSF cytology.

**Administration of our radiolabeled omburtamab via Ommaya reservoir**



**PET scan of distribution of our radiolabeled omburtamab two hours after administration**



The diagram on the left depicts how our radiolabeled omburtamab can be administered via the Ommaya reservoir and catheter into the deep ventricles of the brain where the CSF is produced. From the ventricles, our radiolabeled omburtamab will flow with the CSF and spread throughout the entire

CNS compartment potentially binding and killing B7-H3 positive cancer cells it may find on its way. The diagram on the right is a PET scan showing the distribution of our radiolabeled omburtamab two hours after administration where it has flowed from the central ventricles throughout the entire CNS compartment.

*Primary Objective*

- To define the clinical toxicities of intrathecal <sup>131</sup>I-omburtamab.

*Secondary Objective*

- To collect neurocognitive and long-term follow-up data.

Safety Results

No MTD was reached in the dose escalation portion of the trial. Although not a DLT, myelosuppression was observed in patients who had received craniospinal radiation and <sup>131</sup>I-omburtamab at dose levels six and seven (60 and 70 mCi, respectively). As a result, a dose of 50 mCi was chosen for the expansion cohort. Among the 93 patients treated with <sup>131</sup>I-omburtamab, a total of 293 injections were administered and myelosuppression was observed in approximately 83 patients.

*Long-term toxicities:* There were no significant long-term toxicities directly attributed to <sup>131</sup>I-omburtamab. There was no increased risk of radionecrosis; specifically, neurologic deficits secondary to radionecrosis have not been observed in long-term survivors. However, among long-term survivors with a history of prior high dose induction chemotherapy, myeloablative regimens, craniospinal radiation therapy and <sup>131</sup>I-omburtamab, observed toxicity included short stature and growth hormone deficiency (n=11), hypothyroidism (n=11), cataracts (n=2), persistence of a seizure disorder since CNS NB onset (n=1), and one patient with both an osteochondroma and meningioma (n=1). Unrelated to omburtamab, there were four long-term events causing death in patients who were otherwise in remission due to infection (n=1), pulmonary fibrosis (n=1), and treatment related mortality for secondary leukemia (n=2). Cognitive deficits were noted in three infants who received additional tutorial assistance in school.

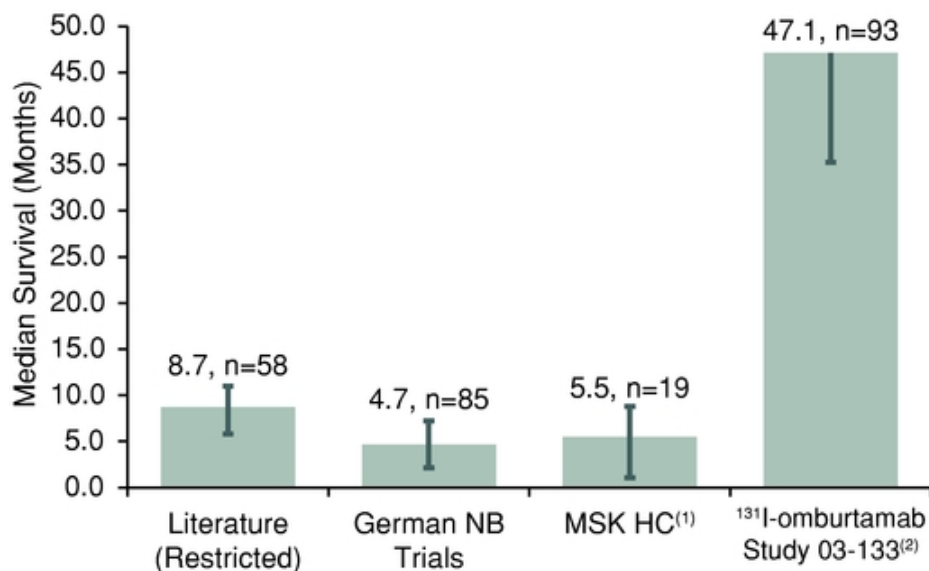
Eighty-one SAEs were reported in CNS/LM from NB patients of which 44 were definitely, possibly or probably treatment-related SAEs. Among the 44 treatment-related SAEs, 36 were Grade 4, six were Grade 3 and two were Grade 2. The most common SAEs were hematological, including BM suppression.

Efficacy Results

Data reported as of August 2017 indicates that the median OS for the 93 patients with R/R NB who have CNS or LM at relapse treated under Study 03-133 was 47 months. Of these 93 patients, 51, or approximately 55%, remained alive from the time of CNS/LM from NB relapse. We believe that the median OS may continue to increase. Based on calculations per the Kaplan-Meier Plot, the estimated three-year OS is 56% and the estimated five-year OS is 43%.

In a previous presentation of ASCO, an analysis of 80 patients showed that 38 patients died. Twenty, or approximately 53%, of these patients were attributed to reasons unrelated to any recurrence of CNS/LM from NB disease. We believe this is further indication of the potential effectiveness of <sup>131</sup>I-omburtamab in treating CNS/LM from NB.

**Comparison of Median Overall Survival (Months)**



(1) MSK HC = NB patients with CNS / LM treated at MSK prior to 2003.

(2) <sup>131</sup>I-omburtamab = Patients with CNS / LM treated under Study 03-133.

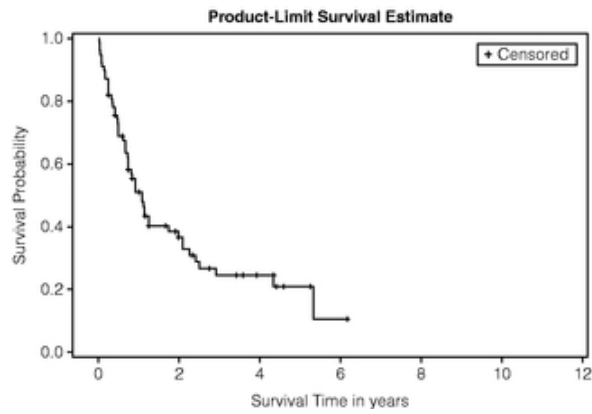
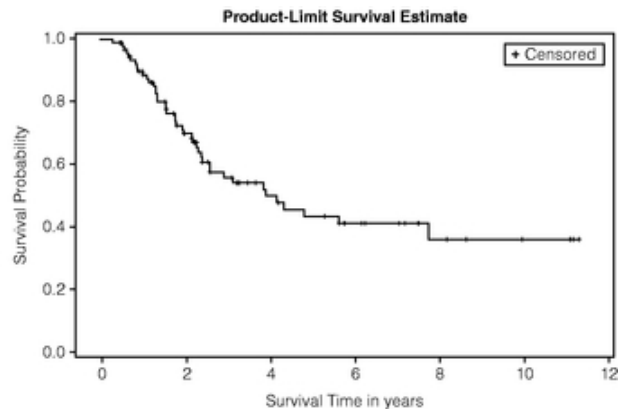
The figure above compares median OS data from Study 03-133 with historical controls (described previously). Historical patient data extracted from three sources revealed median OS of 8.7 months in the literature, 4.7 months in the German NB Trials, and 5.5 months in the MSK historical cohort prior to the introduction of <sup>131</sup>I-omburtamab treatment. These results further demonstrate the lack of an established, effective therapy for these patients that we believe can potentially be addressed by <sup>131</sup>I-omburtamab.

The Kaplan Meier chart below to the left shows the historical comparable data from the restricted analysis of our recent review of published literature discussed above. Of the 58 patients, there were only three cases of survival beyond three years.

The Kaplan Meier chart below to the right shows median OS following the introduction of <sup>131</sup>I-omburtamab treatment. This represents 93 treated patients from Study 03-133 as at August 2017. The estimated three-year median OS was 56% and the five-year median OS was 43%. Survivors have been followed for up to 11.1 years, with a current mean duration of follow up of 2.6 years. Fifty-one, or approximately 55%, of the 93 patients treated with <sup>131</sup>I-omburtamab remained alive at their last follow up.



Historical Cases (n=58)

 $^{131}\text{I}$ -omburtamab (n=93)

*Study 101: A Multicenter Phase 2/3 Trial of the Efficacy and Safety of Intracerebroventricular Radioimmunotherapy using  $^{131}\text{I}$ -omburtamab for Neuroblastoma Central Nervous System/Leptomeningeal Metastases*

Study 101 is a pivotal Phase 2/3 single-arm, open-label, non-randomized, multi-center efficacy, safety, pharmacokinetics and dosimetry trial of intracerebroventricular  $^{131}\text{I}$ -omburtamab in pediatric patients with R/R NB who have CNS or LM. Patients will receive up to two cycles of  $^{131}\text{I}$ -omburtamab. This study is expected to commence in the first quarter of 2018, and we plan to treat an initial 18 patients for BLA submission purposes. The purpose of the study is to demonstrate pharmacokinetic and dosimetry comparability between study sites using  $^{131}\text{I}$ -omburtamab from our cGMP commercial manufacturer and drug product previously produced by MSK. Study 101 has also been designed to satisfy the confirmatory study and post-marketing requirement by the FDA, and as a result, we will continue to recruit 14 more patients in addition to the initial 18 patients. We expect to submit the BLA for CNS/LM from NB in pediatric patients and expect to complete this submission in the second half of 2018.

An interim analysis will be performed when 18 patients have completed evaluations at week six, at which dosimetry and pharmacokinetics objective and available safety and efficacy data will be assessed. Data from this analysis will also be combined with the data from Study 03-133 to support a potential accelerated approval for  $^{131}\text{I}$ -omburtamab for the treatment of pediatric patients with R/R NB who have CNS or LM.

Safety and efficacy data will be investigated with short-term follow-up at 26 weeks after treatment and with long-term follow-up for up to three years following treatment. Final analysis will be performed when all 32 treated patients have completed long-term follow-up (three years or until death).

Median OS at three years and its 95% CI will be estimated using Kaplan-Meier methods. Efficacy will be achieved if the lower limit of the 95% CI of three-year median OS exceeds 10%. PFS will also be analyzed using Kaplan-Meier methods.

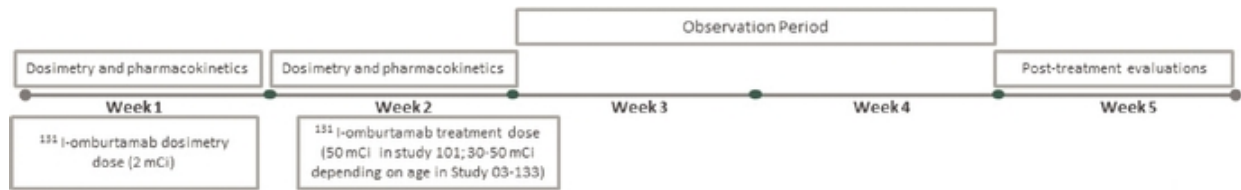
#### Patient Population

In addition to satisfying certain other criteria, patients must be less than 18 years of age at the time of screening. Patients must have a histologically confirmed diagnosis of CNS/LM from NB with relapse.

## Treatment Protocol

A single treatment cycle will last five weeks and will include premedication, intracerebroventricular  $^{131}\text{I}$ -omburtamab administration (one dosimetry dose and one treatment dose), an observation period, and post-treatment evaluations (see figure below).

One  $^{131}\text{I}$ -omburtamab treatment cycle for Study 101



Patients without objective PD are eligible for a second dosing cycle.

### Primary Objective

- To determine OS rate at three years.

### Secondary Objectives

- To determine ORR up to three years.
- To assess PFS at six months after the first therapeutic dose of  $^{131}\text{I}$ -omburtamab.
- To assess radiation doses delivered to the blood and CSF.
- To assess the frequency, type, of adverse events and human anti-mouse antibodies, or HAMA, response formation.
- To assess the effects on cognitive functions.

## $^{124}\text{I}$ -omburtamab for the Treatment of Diffuse Intrinsic Pontine Glioma

$^{124}\text{I}$ -omburtamab is currently being evaluated in an ongoing Phase 1/2 clinical trial (Study 11-011) for the treatment of DIPG. In contrast to Iodine-131, which emits electrons, Iodine-124 is a positron-emitting iodine isotope. This enables measurement of iodine uptake using PET scans, which we believe is important when using radiotherapy in a critical organ such as pons, where overdosing may have serious consequences. In 2016,  $^{124}\text{I}$ -omburtamab received RPDD from the FDA for the treatment of DIPG. As of October 2017, we have treated 33 patients with DIPG with  $^{124}\text{I}$ -omburtamab. Interim clinical results from the dose escalation portion of the study, which were reported at the American Society of Clinical Oncology, or ASCO, in June 2017, demonstrated that convention-enhanced delivery, or CED, of  $^{124}\text{I}$ -omburtamab in the brainstem of children with DIPG appears to be a generally feasible and safe approach for drug delivery, based on an evaluation using distribution and pharmacokinetics. A single dose of 4mCi  $^{124}\text{I}$ -omburtamab was generally considered safe. We plan on reporting additional interim clinical results in the first half of 2018. We believe that we may qualify for a sBLA, assuming positive pivotal data.

### Overview of Diffuse Intrinsic Pontine Glioma

DIPG is a highly aggressive, malignant and difficult to treat brain tumor that forms from the glial (supportive) cells of the brain. The tumor grows in the area of the brainstem, called the pons, a critical area of the brain. Pons are involved in regulating critical body functions such as respiration and consciousness. They also house cranial nerves that facilitate essential functions such as eye movements,

chewing, swallowing, facial expressions, hearing and balance, and assists in the transmission of messages between the various structures of brain and the spinal cord.

DIPG typically affects children between the ages of five to nine years old and is the most common brainstem tumor in children, representing 75% to 80% of pediatric brainstem tumors. There are an estimated 300 children diagnosed with DIPG per year in the United States. One published research analysis evaluating DIPG specimens using immunohistochemistry demonstrated that 100% (nine out of nine) of the tested specimens were B7-H3 positive. While DIPG accounts for approximately 10% to 15% of brain tumors in the pediatric population, it constitutes approximately 80% of brain tumor-related deaths. Assuming similar prevalence as in the United States, we estimate approximately 450 new pediatric patients diagnosed with DIPG per year in Europe.

#### *<sup>124</sup>I-omburtamab for Diffuse Intrinsic Pontine Glioma—Current Treatment Landscape and Associated Limitations*

DIPG grows diffusely and infiltrates healthy tissue in the critical structures of the brainstem and surgical treatment is not possible. The standard of care for the past three decades for children with newly diagnosed DIPG has been focal radiation therapy. Radiotherapy provides temporary improvement or stabilization of symptoms and extends median OS by an average of approximately three months. Within three to eight months after completion of radiation therapy, most children with DIPG have clinical or radiographic evidence of PD. Due to the strong likelihood of the development of PD in the vast majority of children with DIPG, many receive adjuvant chemotherapy at some point during their disease course in an attempt to improve survival. Despite numerous investigational trials, including those evaluating the efficacy of hyperfractionated radiotherapy and high-dose chemotherapy, the limited survival of patients with DIPG remains unchanged.

The prognosis for DIPG remains very poor and the median OS of children with DIPG is less than one year from diagnosis and no meaningful improvement in median OS has been realized in more than three decades. The prognosis for children with DIPG is significantly worse than that of other brainstem tumors.

#### *<sup>124</sup>I-omburtamab for Diffuse Intrinsic Pontine Glioma—Clinical Development Program*

<sup>124</sup>I-omburtamab is currently being evaluated in an ongoing Phase 1 clinical study (Study 11-011) for the treatment of DIPG and we plan on reporting additional clinical results in 2018.

#### *Study 11-011: A Phase I Study of Convection-Enhanced Delivery of <sup>124</sup>I-omburtamab for Patients with Non-Progressive Diffuse Pontine Gliomas Previously Treated with External Beam Radiation Therapy*

MSK is conducting a Phase 1, dose escalation study of CED of <sup>124</sup>I-omburtamab in children with non-progressive DIPG previously treated with external beam radiation therapy. The study commenced in December 2011 and as of October 2017, 33 patients had been enrolled. We expect to enroll a total of 64 patients.

#### Patient Population

In addition to satisfying certain other criteria, patients must be two years of age or older, and 21 years of age or younger. Patients must have non-PD DIPG previously treated with external beam radiation therapy. At least four weeks but not more than 14 weeks must have elapsed from the completion of radiotherapy.

## Treatment Protocol

The intervention is a surgical procedure using interstitial infusion of  $^{124}\text{I}$ -omburtamab into the brainstem tumor. It is performed by stereotactic placement of a small caliber infusion cannula into the tumor followed by a slow infusion CED of  $^{124}\text{I}$ -omburtamab, which was initially administered at doses ranging from 0.25 mCi to 4.0 mCi. Study 11-011 was subsequently amended for further dose escalation cohorts (using 6, 8, 10 and 12 mCi/injection, respectively).

### *Primary Objective*

- To determine the MTD of  $^{124}\text{I}$ -omburtamab administered via interstitial infusion in patients with DIPG.

### *Secondary Objectives*

- To estimate tissue radiation doses and volumes of therapeutic distribution following  $^{124}\text{I}$ -omburtamab interstitial infusion in the brainstem.
- To assess the toxicity profile associated with  $^{124}\text{I}$ -omburtamab administered via CED to the brainstem.
- To analyze OS.
- To explore radiological parameters such as magnetic resonance, or MR, spectroscopy and delta T2 as potential indicators of response.
- To explore lesion dosimetry estimates obtained from serial PET/CT or PET/MR with clinical profile, performance status score and OS.

## Safety Results

As noted above, interim data was presented at the June 2017 annual meeting of ASCO, which demonstrated that CED appears to be a feasible and safe approach for drug delivery in the brainstem of children with DIPG as evaluated using distribution and pharmacokinetics. A single dose of 4 mCi  $^{124}\text{I}$ -omburtamab was considered generally safe.

The principal investigator for Study 11-011, in collaboration with the Pediatric Brain Tumor Consortium, is currently drafting a feasibility study to expand the experiences from Study 11-011 to other sites. This study will be a non-randomized, multi-center, feasibility trial using CED in the brainstem of children with DIPG. Each patient will have previously received external beam radiotherapy to the brainstem and will not have shown clear evidence of tumor progression following this therapy. Diagnostic and eligibility decisions for patients entering the study will be made by a multidisciplinary pediatric neuro-oncology team at the treating site. Eligibility and surgical planning will be centrally reviewed. Patients will undergo a single treatment using CED of  $^{124}\text{I}$ -omburtamab (4mCi). MRI and PET will be used for confirmation of appropriate drug distribution patterns. Perioperative morbidity, device performance (catheter for antibody delivery in pons), and patient tolerance after CED treatment will be monitored. OS and time to recurrence will be monitored. Advanced MR-based algorithms will be used to monitor for geometric response. Serial liquid biopsies (serum, urine, CSF) will be explored as a correlate of tumor response.

### ***$^{131}\text{I}$ -omburtamab for Treatment of Desmoplastic Small Round Cell Tumor***

$^{131}\text{I}$ -omburtamab is currently being evaluated in an ongoing Phase 1 clinical study (Study 09-090) for the treatment of DSRCT. Preliminary data from 34 out of the planned 45 patients demonstrated that treatment with  $^{131}\text{I}$ -omburtamab was generally safe and well tolerated by the patients, as assessed by the trial investigators. No DLTs were observed and a MTD was not reached. In

addition, there was no significant myelosuppression and stem cell rescue was not required. We plan on reporting additional interim clinical results in the first half of 2018. We believe that we may qualify for a sBLA, assuming positive pivotal data.

#### *Overview of Desmoplastic Small Round Cell Tumor*

DSRCT is a rare and aggressive type of a soft tissue cancer (sarcoma) that primarily affects children and young adults and is more common in males. It is formed by small, round cancer cells surrounded by scar-like tissue and is often found in the peritoneum (the tissue that lines the inside of the abdomen and pelvis). Most patients present with abdominal or pelvic tumors, with subsequent metastases to distant lymph nodes, BM and lungs. Due to the rarity of this neoplasm, no large population based studies exist. Analysis presented in literature suggests there are approximately 100 patients diagnosed with DSRCT per year in the United States. Assuming similar prevalence as in the United States, we estimate approximately 150 patients diagnosed with DSRCT per year in Europe. A published report examining DSRCT samples using immunohistochemistry showed that 44 of 46 (or 96%) of tumor samples were B7-H3 positive.

#### *<sup>131</sup>I-omburtamab for Desmoplastic Small Round Cell Tumor—Current Treatment Landscape and Associated Limitations*

Patients are typically managed with aggressive multimodal therapy, including neoadjuvant chemotherapy, maximal surgical debulking, intraperitoneal, or IP, chemotherapy in some cases, adjuvant whole abdominopelvic radiation therapy, and stem cell or BM transplant. Studies have shown that use of intense alkylator therapy and gross total resection have been associated with limited improvements in patient survival; thus, there is still a significant unmet clinical need. Because DSRCT most commonly presents as a multicentric abdominal mass, complete upfront resection is not often possible. DSRCTs are chemosensitive, but often recur, necessitating multimodality therapy with radiotherapy, surgery, and/or high dose chemotherapy with stem cell rescue. Additionally, research shows that with a five-year OS rate of less than 15%, patients almost invariably relapse.

Although many strategies have been attempted, survival in patients with DSRCT remains poor. A review of the published research, including two retrospective studies performed by MSK, suggests that the median OS of DSRCT patients ranges from 17 to 25 months.

#### *<sup>131</sup>I-omburtamab for Desmoplastic Small Round Cell Tumor—Clinical Development Program*

Currently, <sup>131</sup>I-omburtamab is being evaluated in an ongoing clinical study (Study 09-090) for the treatment of DSRCT. After completing the BLA submission for CNS/LM from NB, we intend to discuss with the FDA the protocol for the continuation and expansion of this DSRCT study. We believe that we may qualify for a sBLA, assuming positive pivotal data.

#### *Study 09-090: Phase I Study of Intraperitoneal Radioimmunotherapy with <sup>131</sup>I-omburtamab for Patients with Desmoplastic Small Round Cell Tumors and Other Solid Tumors Involving the Peritoneum*

MSK is conducting a clinical study of IP <sup>131</sup>I-omburtamab for treatment of patients with DSRCT and other B7-H3 positive solid tumors metastatic to the peritoneum. The primary purpose of the study is to define the toxicity and the MTD, assess the pharmacokinetics, and assess response of DSRCT and other solid tumors. The study commenced in April 2010 and as of October 2017, 47 patients had been enrolled.

Patient Population

In addition to satisfying certain other criteria, patients must be over one year old and able to cooperate with radiation safety restrictions during therapy period. Patients must have a diagnosis of  $^{131}\text{I}$ -omburtamab reactive DSRCT or solid tumors that involve the peritoneum.

Treatment Protocol

The study was designed as an open-label single-arm dose escalation study to evaluate IP  $^{131}\text{I}$ -omburtamab, which was administered at doses ranging from 30 mCi/m<sup>2</sup> to 90 mCi/m<sup>2</sup>. The expansion cohort comprised an additional 10 patients who were dosed at 80 mCi/m<sup>2</sup>.

*Primary Objective*

- To define the toxicity and the MTD of IP  $^{131}\text{I}$ -omburtamab.

*Secondary Objectives*

- To assess pharmacokinetics for IP  $^{131}\text{I}$ -omburtamab.
- To assess response of DSRCT and other solid tumors to IP  $^{131}\text{I}$ -omburtamab.

Safety Results

Preliminary results from the 34 patients demonstrated that treatment with  $^{131}\text{I}$ -omburtamab was generally well tolerated by the patients as assessed by the trial investigators. No DLTs were observed and a MTD was not reached. In addition, there was no significant myelosuppression and stem cell rescue was not required. We believe that the initial data from the first group of patients supports continued investigation of the benefit of  $^{131}\text{I}$ -omburtamab in this patient population.

The table below presents safety data from Study 03-133 conducted from January 2004 through August 2017. The outlined information in the below table refers to patients treated in Study 03-133.

**Omburtamab—Clinical Overview**  
**Patient Profile and AEs (Jan 2004 - Aug 2017)**

Cancer Diagnosis	No. of Patients	No. Injections	Adverse Event (CTC 3.0) Possibly or Probably	Percent Myelosuppression (Gr 3 or 4)
Neuroblastoma	93	293	Gr 3 or 4 myelosuppression (ANC, hgb, platelets) (83) Gr 4 Hypersensitivity reaction (1) Gr 3 ALT/AST (5) Gr 3 Chemical Meningitis (3) Gr 4 MDS/AML (5)	89%
Medulloblastoma/ PNET	15	29	Gr 3 or 4 myelosuppression (6) Gr 4 chemical meningitis (1)	43%
Ependymoma	9	37	Gr 3 or 4 myelosuppression (3)	33%
EMTR	2	4	Gr 3 or 4 myelosuppression (2)	100%
Sarcoma	6	18	Gr 3 or 4 myelosuppression (3) Gr 4 AML (1)	50%
Melanoma	4	9	Gr 3 myelosuppression (2) Gr 3 nausea (1) Gr 3 hypokalemia (1)	50%
Other <sup>(1)</sup>	5	22	Gr 4 MDS/AML (1)	
<b>Total</b>	<b>134</b>	<b>412</b>		

(1) Includes ATRT, choroid plexus cancer, ovarian cancer, retinoblastoma.

**Non-Clinical Safety**

The non-clinical studies evaluating the pharmacology and toxicology of omburtamab have demonstrated that omburtamab has an acceptable safety profile, with no significant toxicity observed in different species, including rats and non-human primates. Omburtamab has preferential affinity for a spectrum of cancerous tissues that express B7-H3, with minimal binding to normal tissues. Omburtamab specifically targets the membrane of cancer cells. We believe that the lack of cross-reactivity with normal human tissue, specifically within the brain, and the localized binding of omburtamab to the membranes of cancer cells that express B7-H3, makes omburtamab a viable candidate for a targeted radiotherapy.

The safety and feasibility of dosing with either naked or radiolabeled omburtamab has been demonstrated using different routes of administration and in different species. In mice, rats, and monkeys naked and/or radiolabeled omburtamab appeared to be well-tolerated when administered intravenously, intracerebroventricularly, or via interstitial infusion into the brain parenchyma. Furthermore, since omburtamab does not react with normal human brain tissue, intracerebroventricular treatment with omburtamab has been shown to be generally well tolerated by human pediatric patients in a number of clinical trials.

**Omburtamab—DTPA Overview**

We intend to leverage our expertise with omburtamab to develop product candidates for the treatment of indications associated with larger adult patient populations. We believe that our clinical experience with <sup>131</sup>I-omburtamab in 41 patients with tumors such as sarcoma, melanoma and medulloblastoma supports this objective. Our first such product candidate targeted towards larger patient populations is DTPA-conjugated omburtamab radiolabeled with Lutetium-177, which is currently

in pre-clinical development for the treatment of B7-H3 positive LM from solid tumors. DTPA (diethylenetriamine pentaacetate) is an organic molecule that acts as a chelator of metals such as Lutetium. DTPA can bind to radioactive materials to decrease the amount of time it takes to flush the radioactive material from the body. The resulting product candidate, omburtamab-DTPA-Lutetium-177 conjugate, or <sup>177</sup>Lu-omburtamab-DTPA, can be distributed directly to hospitals, already conjugated and ready to use. It may then be administered to patients as a single-step push dose via an Ommaya reservoir, similar to the administration of <sup>131</sup>I-omburtamab in CNS/LM from NB. We believe this is an important advantage because radiopharmacies within hospitals have limited capacity for radiolabeling. Therefore, we believe that a more easily available ready to use radiolabeled antibody such as <sup>177</sup>Lu-omburtamab-DTPA could be used more frequently, thereby significantly expanding our patient population beyond children. We expect to file an IND for <sup>177</sup>Lu-omburtamab-DTPA for treatment of B7-H3 positive LM from solid tumors in late 2018 or early 2019.

### ***Overview of B7-H3 Positive Central Nervous System/Leptomeningeal Metastases from Solid Tumors***

As previously described, CNS/LM is a rare and usually fatal complication of cancer in which the disease spreads to the membranes, or meninges, surrounding the brain and spinal cord. The incidence of metastatic brain tumors is estimated to be 200,000 to 300,000 people per year. Studies have shown that the most common tumors which metastasize to the brain express B7-H3.

Although any cancer can metastasize to the leptomeninges, breast cancer (12% to 35%), lung cancer (10% to 26%), melanoma (5% to 25%), gastrointestinal malignancies (4% to 14%), and cancers of unknown primary (1% to 7%) are the most common causes of solid-tumor-related LM. We believe that the annual incidence of CNS/LM across all tumor types is at least 30,000 patients in the United States and Europe combined.

Despite aggressive treatment, CNS/LM has a poor prognosis with less than 15% of all patients surviving one year following diagnosis. The median OS of untreated patients with CNS/LM is four to six weeks. The median OS of patients with combined treatment (often comprising surgery, radiation and/or chemotherapy) is usually less than eight months.

The incidence of CNS/LM is increasing. An important factor contributing to the increasing incidence of CNS/LM is the availability of more effective systemic therapies. These therapies may increase survival time and could therefore lead to a higher incidence of metastatic disease.

### ***<sup>177</sup>Lu-Omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases—Current Treatment Landscape and Associated Limitations***

Treatment of most patients with CNS/LM requires a combination of surgery, radiation, and/or chemotherapy. However, CNS/LM has been proven difficult to treat due to the localization of the tumor within the CNS compartment making complete removal by surgery difficult. Moreover, the blood-brain barrier, a membrane that selectively regulates molecules entering the brain from the blood, often inhibits drug delivery to the brain due to the inability of large molecules to cross the blood-brain barrier. Because the most common tumors that metastasize to the brain express B7-H3, in contrast with normal brain tissue that lacks B7-H3 expression, we believe that the incidence of B7-H3 expression makes omburtamab a viable antibody for targeting metastatic tumors in the CNS.

### ***<sup>177</sup>Lu-Omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases—Mechanism of Action***

We are developing a Lutetium-177 conjugated omburtamab with DTPA as chelator. <sup>177</sup>Lu-omburtamab-DTPA will be given as a single-step push dose administration to patients. The administration for CNS/LM will be intrathecal via an Ommaya reservoir similar to the administration of <sup>131</sup>I-omburtamab in CNS/LM from NB. This form of administration will allow us to bypass the blood



brain barrier and gain direct access to the CNS/LM. Lutetium-177 is a medium-energy beta-emitter with a maximal tissue penetration of 2 mm. Its half-life is approximately 6.7 days. Lutetium-177 also emits low-energy Gamma rays, which allows scintigraphy and subsequent dosimetry with the same therapeutic compound. Lutetium-177 is bound to omburtamab by DTPA. The resulting product <sup>177</sup>Lu-omburtamab-DTPA conjugate can be distributed conjugated ready to use. Lutathera, a Lutetium-177-DOTA conjugated to an analogue of somatostatin, has already demonstrated significant clinical efficacy in patients with progressive neuro endocrine tumors, or NETs, and is approved by the EMA, and is currently under review for approval by the FDA, in this orphan indication. In a multi-center, randomized, comparator-controlled, parallel-group Phase 3 study that has been the basis for regulatory submission for Lutathera, it demonstrated a significant improvement in PFS in patients with inoperable progressive midgut NETs compared to the general standard of care, with limited acute toxic effects. The beta radiation of Lutetium-177 is similar to the beta radiation emitted from radioactive iodine, which already has demonstrated efficacy in CNS/LM from NB when conjugated to omburtamab.

We believe Lutetium-177 may have a number of potential advantages over both Iodine-131 and Iodine-124. In particular, the radiolabeling of omburtamab-DTPA with Lutetium-177 involves a relatively simple one-step procedure and can be distributed conjugated ready to use.

### **Humanized Omburtamab Overview**

We are also developing hu-omburtamab, a humanized version of omburtamab, for the treatment of B7-H3 positive adult solid tumors where systemic immunotherapy is needed. We expect that hu-omburtamab will be used as a radio-conjugated antibody designed to overcome limitations of murine antibodies that may induce HAMA, which may lead to decreased efficacy and increased toxicity when used for systemic immunotherapy.

### **Bispecific Antibody Program Overview**

We are advancing a promising pipeline of bivalent tumor targeting BsAbs for the treatment of cancer. We believe that our BsAbs have the potential to overcome limitations associated with existing BsAb constructs. Our first BsAb product candidate, naxitamab-BsAb, is a humanized anti-GD2 and anti-CD3 BsAb. Together with MSK, in the first half of 2018, we plan to submit an IND in order to commence a Phase 1/2 trial in patients with GD2-positive solid tumors refractory to available therapy.

Our second BsAb product candidate, CD33-BsAb, is a humanized anti-CD33 and anti-CD3 BsAb. We intend to submit an IND in 2019 in order to commence a Phase 1 trial in patients with hematological cancers expressing CD33.

In addition, the MSK License provides us with non-exclusive access to MSK's technology that facilitates the creation of a novel human protein tag that can dimerize, or link together, BiTEs, which we refer to as the MULTI-TAG technology platform. BiTEs are an important class of BsAbs that has shown significant promise in the treatment of cancer due to their high potency. Based on our pre-clinical studies, we believe that this novel class of BiTEs has the potential to result in better tumor-binding, longer serum half-life and significantly greater T-cell mediated killing of tumor cells without the need for continuous infusion. We plan to utilize this technology to create a diverse platform of dimerized BiTEs. We are currently working with several MULTI-TAG candidates with MSK and our goal is to be able to select a clinical candidate in 2018.

#### *Overview of Current Bispecific Antibody Treatment Approaches*

BsAbs are engineered proteins capable of simultaneously binding to two different epitopes, on the same or different antigens. Through simultaneous recognition of two different targets, BsAbs can serve as mediators for the redirection of immune effector cells, such as Natural Killer cells, or NK cells, and T-cells, to tumor cells, in order to enhance tumor cell destruction. In addition, by targeting

two different receptors in combination on the same cell, BsAbs can induce modifications of cell signaling, including the inactivation of pathways. BsAbs represent an exciting approach to cancer immunotherapy because, among other factors, they have the potential to overcome the limitations of conventional monoclonal antibody approaches to treating complex cancers. Moreover, BsAbs can be mass produced without the manufacturing complications and risk of persistent systemic toxicity associated with other new immunotherapy approaches such as CAR-T therapy.

BsAbs are generally divided into two classes, IgG-like molecules and non-IgG-like molecules. IgG-like BsAbs retain the traditional monoclonal antibody structure but bind to multiple antigens. Although IgG-like BsAbs generally demonstrate adequate stability and effector functions, their large size limits tissue penetration.

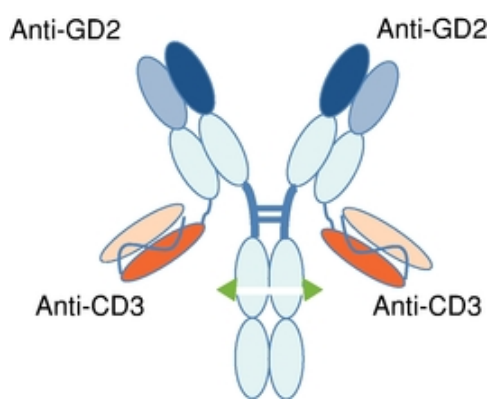
Non-IgG-like BsAbs lack a fragment crystallizable, or Fc, region, consisting instead of chemically linked variable regions and various types of multivalent single-chain variable fragments, or scFvs. One type of non-IgG-like BsAbs are BiTEs. BiTEs are relatively small and have more efficient penetration, however, they exhibit short serum half-lives. They bind monovalently to tumor targets, which often results in suboptimal tumor binding relative to conventional IgG-like BsAbs that bind bivalently. Finally, therapeutic dosing of BiTEs is limited by the risk of excessive cytokine release in patients.

The only approved BsAb in the United States is blinatumomab, a BiTE, approved for the treatment of acute lymphocytic leukemia.

#### *Naxitamab-BsAb Overview*

The figure below depicts our first BsAb product candidate, naxitamab-BsAb, a fully humanized IgG-scFv format antibody, in which the anti-CD3 scFv is linked to the carboxyl end of the naxitamab IgG1 and the Fc region is mutated to help prevent cytokine release as well as complement-mediated pain side effects.

#### **Naxitamab (anti-GD2 and anti-CD3) Bispecific Antibody**



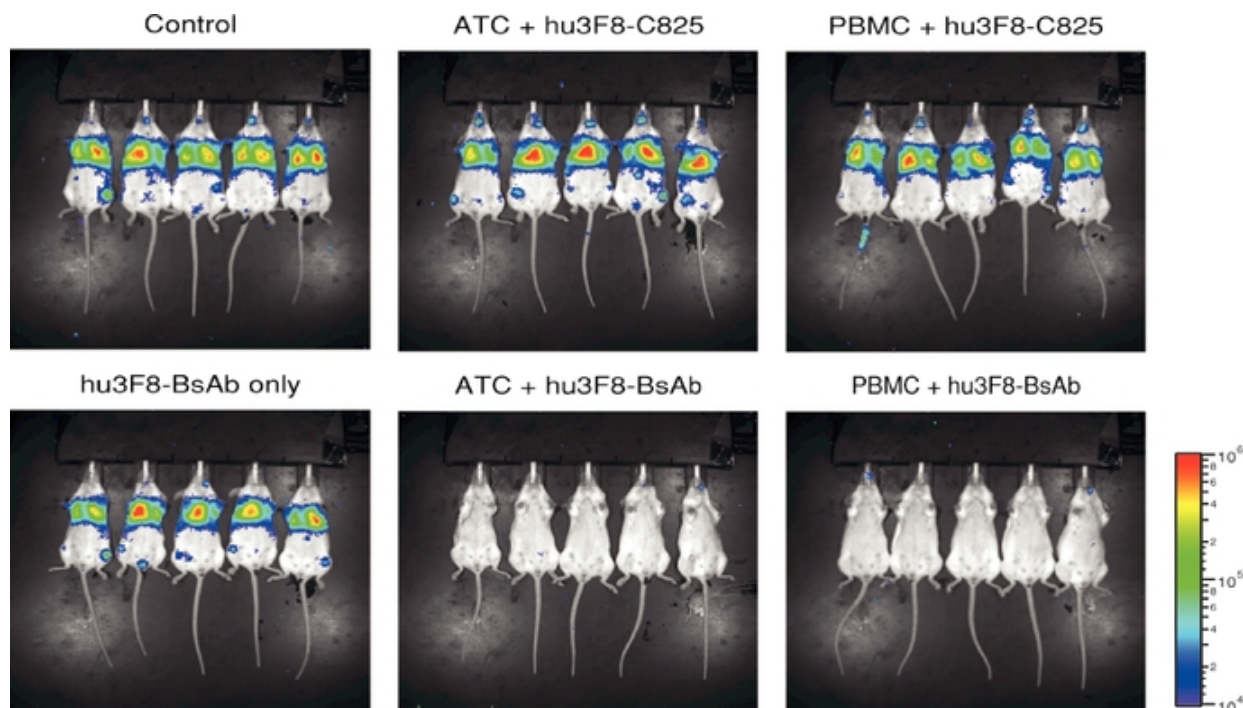
We believe that naxitamab-BsAb may have several potential advantages over other BsAbs, including:

- Improved potency due to bivalency towards GD2, while maintaining functional monovalency towards CD3.
- Longer serum half-life to improve efficacy and patient convenience—molecular size of 210kD (vs. 55kD size of blinatumomab) and binding to neonatal Fc receptor result in longer serum half-life, thereby reducing the need for continuous infusion.

- Better safety profile:
  - The larger size of our molecule prevents leakage into the CNS thereby avoiding CNS neurotoxicity;
  - Low affinity for CD3 molecules and functional monovalency towards CD3 reduces risk of significant cytokine release; and
  - Lower immunogenicity as shown by the low immunogenicity profile of naxitamab.

Knockout mice, which lack murine T-cells, B-cells and NK cells, were used for human cancer xenograft studies. The picture below demonstrates a study where mice were transplanted with human M14-Luc melanoma and human peripheral blood mononuclear cells, or PBMC, or activated T-cells, or ATC, as effector cells. Tumor growth was assessed by luciferin bioluminescence.

Mice, in a control group, treated with saline without effector cells (naxitamab-BsAb only), or effector cells plus ATC+naxitamab-C825, used as the control BsAb and which does not bind to T-cells, had equally rapid tumor progression. In contrast, mice treated with naxitamab-BsAb in the presence of human effector cells (ATC+naxitamab-BsAb or PBMC+naxitamab-BsAb) demonstrated nearly total tumor elimination. The picture below is a representative image at day 31.



Based on this pre-clinical evidence, in 2019, we expect to file an IND to commence a Phase 1/2 trial of naxitamab-BsAb in patients with GD2-positive solid tumors refractory to available therapy.

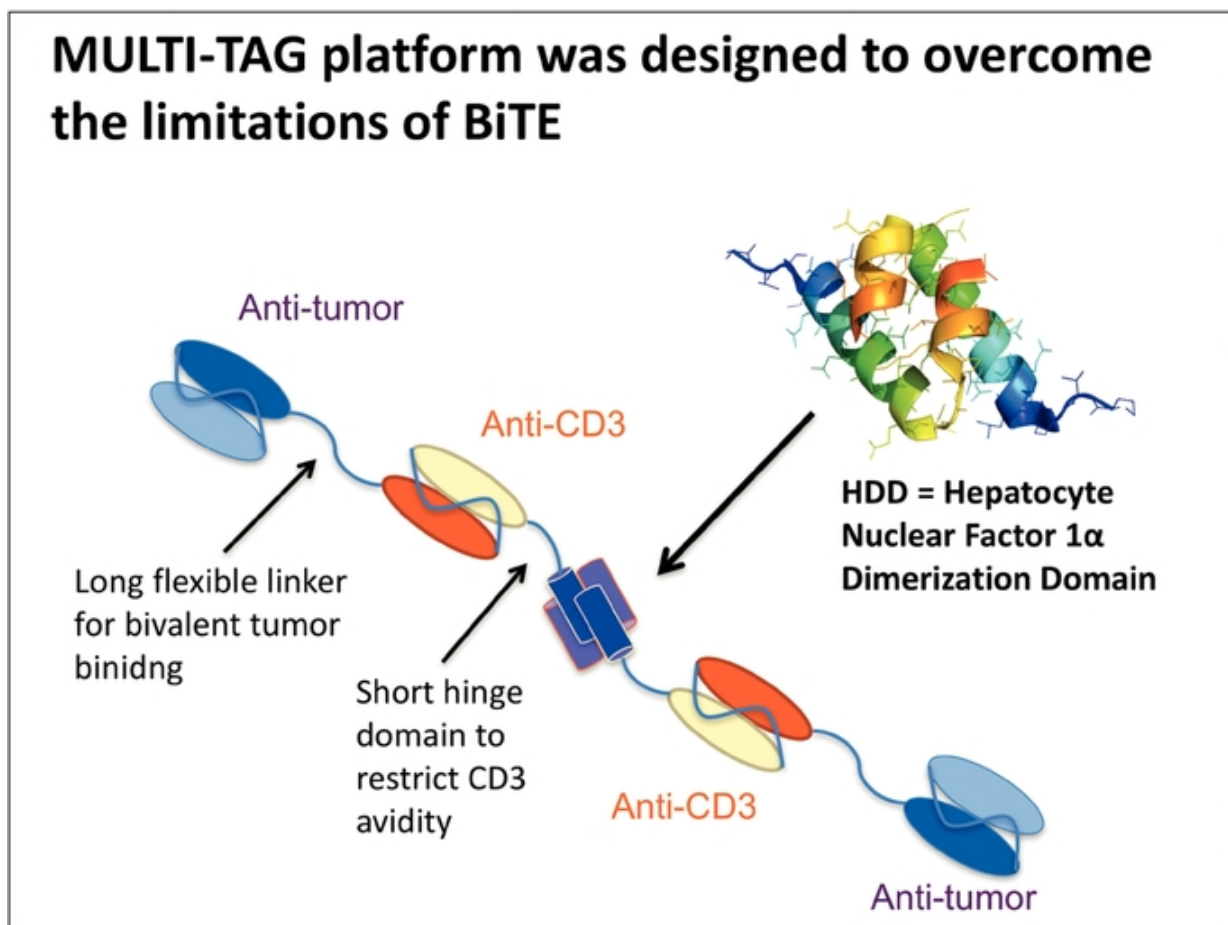
#### *CD33 Overview*

Our second BsAb product candidate, CD33-BsAb, is a humanized anti-CD33 and anti-CD3 BsAb for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. Currently we are planning to set up current Good Laboratory Practices, or GLP, and cGMP production allowing for initiation of formal pre-clinical toxicology in 2018 and potential IND filing in 2019.

We believe that our non-exclusive access to the MULTI-TAG technology will help us make further advances to our BsAb program by optimizing BiTEs. While there has been significant enthusiasm for BiTEs given their high potency and ability to penetrate more efficiently than conventional IgG-like BsAbs, their efficacy remains hampered by their size and binding characteristics. BiTEs are relatively small in size, approximately 55kD, resulting in a short serum half-life given rapid renal clearance. As a result, they require continuous infusion for several weeks in order to achieve a therapeutic response. They also bind monovalently, which often results in suboptimal tumor binding. Further, therapeutic dosing of BiTEs is limited by the risk of excessive cytokine release in patients.

Using the MULTI-TAG technology, we have designed a novel protein tag of human origin that dimerizes, or links, BiTEs, in a unique conformation, which we believe may result in improved tumor binding, a longer half-life, and greater T-cell mediated tumor cell killing. We are using the MULTI-TAG technology platform to dimerize our BsAbs into proteins of approximately 120kD in size, thereby increasing serum half-life without the need for continuous infusion. The unique dimerized conformation, while binding bivalently to tumors, also binds monovalently to T-cells, which we believe, leads to limiting excessive cytokine release. Below is a graphic illustration of the MULTI-TAG technology, to which, under our MSK License, we have unlimited access to use MSK's rights in the technology for any target.

**MULTI-TAG—Dimerization technology to enhance potency of T-cell engaging antibodies**



We are currently working with several MULTI-TAG candidates with MSK and our goal is to be able to select a clinical candidate in 2018.

## **Manufacturing**

Currently, we contract with third party cGMP vendors for the manufacturing of our product candidates for pre-clinical studies and clinical trials and intend to do so in the future, including for commercialization if our product candidates receive marketing approval. We do not currently own or operate any manufacturing facilities for the production of clinical or commercial quantities of our product candidates. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. To meet our projected needs for commercial manufacturing, if the need arises, third parties with whom we currently work may need to increase their scale of production or we may need to secure alternate suppliers. Although we rely on our cGMP manufacturers, we have personnel with substantial manufacturing experience to oversee our relationships with such manufacturers.

Manufacturing clinical products is subject to extensive regulations that impose various procedural and documentation requirements, which govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance. Our vendors are required to comply with cGMP regulations, which are regulatory requirements enforced by the FDA and other regulatory bodies like the EMA to assure proper design, monitoring and control of manufacturing processes and facilities for human pharmaceuticals.

Our current product candidates are mAbs and BsAbs. The manufacturing process for antibodies involves the genetic engineering of a parental host cell line to isolate a cell that produces the antibody. Once the cell or clone (colony of cells derived from a single cell) is isolated, a cell bank is produced under prescribed and documented conditions. The cell bank, preserved frozen, is tested, as required by regulations, to demonstrate that the engineered cell line is free from potentially harmful impurities and contaminants, such as viruses.

The drug substance is an active ingredient that is intended to furnish pharmacological activity or other direct effect in the diagnosis, cure, mitigation, treatment, or prevention of disease or to affect the structure or any function of the human body, but does not include intermediates used in the synthesis of such ingredient while the drug product is a finished dosage form. The manufacturing process for the drug substance begins with the thaw of vials from the cell bank and growth of these cells in established media until sufficient cells are cultured to inoculate a production bioreactor. The cells in the production bioreactor are grown in chemical defined media and under controlled and monitored conditions that stimulate the production of the antibody into the culture media. The production bioreactor is cultured for an established time period and is then harvested by filtration to remove the cells from the culture media.

The antibody solution is purified through a number of steps to remove known process- and product-derived impurities. The technologies employed include ultrafiltration and column and membrane chromatography. Additional steps are performed to inactivate or remove viruses. The final step of the drug substance process adjusts the antibody concentration and produces the final formulation to be used for drug product production. The drug substance is tested to meet pre-established criteria for purity, potency and safety, and is then periodically tested to demonstrate stability upon storage as required by regulations. The drug substance is stored at prescribed temperatures, typically refrigerated or frozen.

The drug product is produced by sterilization filtration of the drug substance solution, followed by aseptic filling into glass vials and then stoppered. The drug product is subjected to release testing for purity, potency and safety according to pre-established specifications. Drug product lots are periodically tested to demonstrate stability over the established storage expiry period. The drug product

is stored and shipped under temperature-controlled conditions, typically refrigerated, to sites designated for clinical trial testing, or eventually to commercial pharmaceutical logistics providers.

Naxitamab is a recombinant humanized IgG1k monoclonal antibody against GD2 expressed in Chinese Hamster Ovary, or CHO, cells. One mL ampoule from the master or working cell bank is used as seeding for a 1000 L fed batch bioreactor in chemical defined media with no animal derived component. After the growths of the cells are completed the un-processed bulk from the bioreactor containing the naxitamab drug substance undergoes conditioned clarified harvests, filtration, and subsequent multi-step product purification.

The naxitamab drug substance is manufactured by Patheon UK Limited in Groningen, The Netherlands and the naxitamab drug product is manufactured at Patheon Manufacturing Services LLC in Greenville, North Carolina, or collectively Patheon, in compliance with cGMP regulations and no excipients of human or animal origin have been used. The naxitamab drug product is packaged in 10 mL ISO 10R glass vials and frozen.

Omburtamab is a murine IgG1 monoclonal antibody against B7-H3. The antibody is manufactured in a 200 L bioreactor in chemical defined media with no animal derived components. After harvests, clarification of the fermentation and a multi-step purification process, the final drug substance is ready for radiolabeling. This non-radiolabeled omburtamab is packaged in 2 mL ISO 2R glass vials and frozen. The drug substance is manufactured by EMD Millipore Corporation, or EMD, in Martillac, France, and the omburtamab drug product is manufactured by Patheon in Ferentino, Italy.

While we believe that Patheon and EMD are capable of producing sufficient quantities of drug product to support our currently planned clinical trials for naxitamab and omburtamab, we also believe that there are a number of alternative third-party manufacturers that have similar capabilities that would be capable of providing sufficient quantities of drug product for our planned clinical trials. However, should Patheon and/or EMD not be able to provide sufficient quantities of drug product for our planned clinical trials, we would be required to seek another contract manufacturer to provide this drug product, likely resulting in a delay in such trials.

### **Commercialization Plan**

The sales call points for our late-stage product candidates in the United States and the European Union are highly concentrated around a few major hospitals and, therefore, can be effectively serviced with a small commercial organization. Both our existing clinical trials at all the relevant sites, as well as our partnership with MSK, have already afforded us the opportunity to identify patients for our product candidates, if approved. We believe these factors position us well for commercialization.

Our management team understands the complexity of rare oncological diseases and we believe we have the necessary expertise to be a true partner to patients, caregivers, and advocacy and healthcare teams leading to shared success. As we advance our product pipeline to address larger patient populations, we intend to establish a specialty sales force and develop an organizational infrastructure to support the network of relevant hospitals, cancer centers, oncologists and other physicians as well as provide support to patients, care-givers and other healthcare providers. We plan to commercialize our future product candidates in the United States and Europe ourselves, and will evaluate strategic collaborations in select territories in order to maximize the potential of our product candidates.

As additional product candidates advance through our pipeline, our commercial plans may change. The size of the development programs, size of the target market, size of a commercial infrastructure, and manufacturing needs may all influence our strategies in the United States, the European Union and other parts of the world.

## **Competition**

The biotechnology and pharmaceutical industries generally, and the cancer drug sector specifically, are characterized by rapidly advancing technologies, evolving understanding of disease etiology, intense competition and a strong emphasis on intellectual property. While we believe that our product candidates and our knowledge and experience provide us with competitive advantages, we face substantial potential competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions and governmental agencies and public and private research institutions.

In addition to the current standard of care for patients, commercial and academic clinical trials are being pursued by a number of parties in the field of immunotherapy. Early results from these trials have fueled continued interest in immunotherapy, which is being pursued by several biotechnology companies as well as by large pharmaceutical companies. Many of our current or potential competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, manufacturing, pre-clinical studies, conducting clinical trials, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Specifically, MacroGenics, Inc. and Daiichi Sankyo Co. are developing antibodies against B7-H3. In addition, United Therapeutics Corporation has commercialized Unituxin (dinutuximab), an antibody against GD2, in the United States and we believe EUSA Pharma (UK) Ltd. plans to commercialize a similar antibody (dinutuximab beta) under the name Isquette in Europe.

## **Intellectual Property**

### ***Patent Portfolio***

We strive to protect and enhance the proprietary technology, inventions, and improvements that we believe are commercially important to our business, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from our collaborators or other third parties. Our policy is to seek to protect our proprietary position by, among other methods, filing patent applications in the United States and in jurisdictions outside of the United States related to our proprietary technology, inventions, improvements, and product candidates that are important to the development and implementation of our business. We also rely on trade secrets and know-how relating to our proprietary technology and product candidates, continuing innovation, and in-licensing opportunities to develop, strengthen, and maintain our proprietary position in the field of immunotherapy. We additionally rely on data exclusivity, market exclusivity, and patent term extensions when available, and plan to seek and rely on regulatory protection afforded through orphan drug designations. Our commercial success will depend in part on our ability to obtain and maintain patent and other proprietary protection for our technology, inventions, and improvements, whether developed internally or licensed from our collaborators or other third parties; to preserve the confidentiality of our trade secrets; to maintain our licenses to use intellectual property owned by third parties; to defend and enforce our proprietary rights, including our patents; and to operate without infringing on the valid and enforceable patents and other proprietary rights of third parties.

We have in-licensed numerous patents and patent applications and substantial know-how relating to the development and commercialization of our immunotherapy product candidates, including related manufacturing processes and technology. These in-licensed patents and patent applications



claim the inventions of investigators at MSK, as described in more detail in the section herein entitled "Business—Intellectual Property—MSK Agreements."

As of December 31, 2017, our patent portfolio included:

- For our naxitamab patent portfolio, we have an exclusive license from MSK to MSK's rights in two patent families. The first family consists of patents and patent applications with composition of matter claims covering humanized or chimeric antibodies or fragments thereof comprising specific sequences and capable of binding to GD2, and includes two U.S. patents, one Australian patent, two New Zealand patents, one Chinese patent, one Japanese patent, one pending patent application in the United States and six pending patent applications in other jurisdictions, including Europe, Canada, Japan, South Korea, Hong Kong and India. We expect that any patents that issue in this first family will expire in June 2031. The second family consists of applications with composition of matter claims covering high affinity anti-GD2 antibodies, and includes one pending patent application in the United States and nine pending patent applications in other jurisdictions, including Europe, Canada, Australia, China, Japan, South Korea, Hong Kong, Brazil and Russia. We expect that any patents that issue in this second family will expire in March 2034.
- For our omburtamab patent portfolio, we have an exclusive license from MSK to MSK's rights in two patent families. The first family consists of patents and patent applications with composition of matter claims covering antibodies produced by a distinct hybridoma cell line, antibodies comprising specific sequences, polypeptides comprising specific sequences, and process claims covering a method of inhibiting the growth of tumor cells, a method for imaging a tumor in a subject and a method for treating a mammalian subject, and includes seven U.S. patents, one German patent, one Spanish patent, one French patent, one patent in Great Britain, one Italian patent, two Canadian patents, one pending patent application in the United States and one pending patent application in Europe. We expect that any patents that issue in this first family will expire between October 2021 and January 2026. The second family consists of patents and patent applications with process claims covering a method of improving the prognosis or prolonging the survival of a subject bearing a tumor, and includes one Chinese patent, one Indian patent, and two pending patent applications in other jurisdictions, including Europe, and Canada. We expect that any patents that issue in this second family will expire in March 2028.
- For our hu-omburtamab patent portfolio, we have an exclusive license from MSK to MSK's rights in one patent family consisting of patent applications with composition of matter claims covering antibody agents that bind specifically to protein 2Ig-B7H3 or 4Ig-B7H3, and includes one pending patent application in the United States and 12 pending patent applications in other jurisdictions, including Europe, Canada, Australia, New Zealand, China, Japan, South Korea, Eurasia, India, Brazil, South Africa, and Hong Kong. We expect that any patents that issue in this family will expire in August 2035.
- Our Multimerization Technology patent portfolio, which *inter alia* relates to naxitamab-BsAb, includes one patent family under which we have a partly exclusive license to MSK's rights in the patent application. The license is exclusive for MSK's rights in the patents rights of this family that claim products, such as bispecific antibodies which are also claimed by other patent rights licensed from MSK, and non-exclusive for patents rights of this family that claim a product that is not claimed by another patent right licensed from MSK. This family consists of patents and patent applications with composition of matter claims covering bispecific binding agents comprised of two fusion proteins, and includes one U.S. patent, one pending patent application in the United States and nine pending patent applications in other jurisdictions, including Europe, Canada, Australia, China, Japan, South Korea, Hong Kong, Russia and Brazil. We expect that any patents that issue in this first family will expire in March 2034.



- Our CD33 antibody patent portfolio, which includes one patent family under which we have an exclusive license from MSK to MSK's rights in the patent application. This family consists of one U.S. provisional patent application relating to anti Siglec-3 (CD33) antibodies generated from a specific principal investigator's laboratory at MSK. We expect that any patents that issue in this family will expire in April 2038, assuming a future Patent Cooperation Treaty filing.

The term of individual patents depends upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, the patent term is generally 20 years from the earliest claimed filing date of a non-provisional patent application in the applicable country. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the United States Patent and Trademark Office, or USPTO, in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over a commonly owned patent or a patent naming a common inventor and having an earlier expiration date. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration date of a U.S. patent as partial compensation for the length of time the drug is under regulatory review while the patent is in force. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. We cannot provide any assurance that any patent term extension with respect to any U.S. patent will be obtained and, even if obtained, what the duration of such extension may be.

Similar provisions are available in the European Union and certain other non-U.S. jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our product candidates receive approval by the FDA or non-U.S. regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical trials for each drug and other factors. The expiration dates referred to above are without regard to potential patent term extension or other market exclusivity that may be available to us. However, we cannot provide any assurances that any such patent term extension of a non-U.S. patent will be obtained and, even if obtained, the duration of such extension.

As for the immunotherapy products and processes we develop and commercialize, in the normal course of business, we intend to pursue, when possible, composition, method of use, dosing and formulation patent protection. We may also pursue patent protection with respect to manufacturing and drug development processes and technology.

Individual patents extend for varying periods of time, depending upon the date of filing of the patent application, the date of patent issuance, and the legal term of patents in the countries in which they are obtained. Generally, patents issued for applications filed in the United States are effective for 20 years from the earliest effective filing date. In addition, in certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of the FDA regulatory review period. The restoration period cannot be longer than five years and the total patent term, including the restoration period, must not exceed 14 years following FDA approval. The duration of patents outside of the United States varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date. Generally, as noted above, our in-licensed issued patents in all jurisdictions will expire on dates ranging from 2021 to 2031. If patents are issued on our pending patent applications, the resulting patents are projected to expire on dates ranging from 2021 to 2035 (2038 assuming the future filing of a priority claiming Patent Cooperation Treaty application). However, the actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its

coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country, and the validity and enforceability of the patent.

### **Trademarks**

We have filed an application with the USPTO to secure trademark registration for the "Y-mAbs" mark. We currently rely on our unregistered trademarks, trade names and service marks, as well as our domain names and logos, as appropriate, to market our brands and to build and maintain brand recognition. We are seeking to register and will continue to seek to register and renew, or secure by contract where appropriate, trademarks, trade names and service marks as they are developed and used, and reserve, register and renew domain names as appropriate. However, we have not yet registered any of our trademarks, trade names or service marks with the USPTO. If we do not secure successfully register trademark registration for our trademarks, including the "Y-mAbs" mark, we may encounter difficulty in enforcing, or be unable to enforce, our rights in our trademarks, trade names and service marks against third parties.

### **Trade Secrets**

We may also rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets are difficult to protect. We seek to protect our technology and product candidates, in part, by entering into confidentiality agreements with those who have access to our confidential information, including our employees, contractors, consultants, collaborators, and advisors. We also seek to preserve the integrity and confidentiality of our proprietary technology and processes by maintaining physical security of our premises and physical and electronic security of our information technology systems. Although we have confidence in these individuals, organizations, and systems, agreements or security measures may be breached and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or may be independently discovered by competitors. To the extent that our employees, contractors, consultants, collaborators, and advisors use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions. For this and more comprehensive risks related to our intellectual property and proprietary technology, inventions, improvements and products, please see the section on "Risk Factors—Risks Related to Intellectual Property."

### **MSK Agreements**

On August 20, 2015, we entered into the MSK License, which grants us a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments. The MSK License is exclusive with respect to MSK rights in such patent rights and tangible materials within such know-how, and nonexclusive with respect to MSK's rights in such know-how and related intellectual property rights. The patents and patent applications covered by the MSK License are directed, in part, to the naxitamab and omburtamab antibody families, including humanized and chimeric antibodies, as well as MSK's rights in BsAbs, compositions, and their respective use for immunotherapy. Upon entering into the MSK License in 2015 and in exchange for the licenses thereunder, we paid to MSK an upfront payment of \$500,000, issued 1,428,500 shares of our common stock to MSK and agreed to provide certain anti-dilution rights to MSK as further described below. In addition, we are required to pay to MSK certain royalty and milestone payments. We recorded a total expense of \$285,700 for the shares of common stock issued to MSK in 2015 based on the estimated fair value of the shares of common stock of \$0.20 per share at issuance date.

Pursuant to our MSK License and MSK CD33 License, as of December 31, 2017, we have rights to approximately 10 issued U.S. patents, approximately six pending U.S. patent applications, and

other patents and patent applications in jurisdictions outside the United States. Upon entering the MSK License, we made an upfront payment to MSK, and we are required to make to MSK certain royalty payments, including minimum annual royalty payments commencing on the fifth anniversary of the MSK License, which are fully creditable against earned royalties.

The MSK License requires us to pay to MSK mid to high single-digit royalties based on annual net sales of licensed products or the performance of licensed services by us and our affiliates and sublicensees. We are required to pay annual minimum royalties of \$80,000 over the royalty term, starting in 2020, which amounts are non-refundable but are creditable against royalty payments otherwise due thereunder. Total expensed minimum royalty payments under the MSK License were \$1,200,000 in 2016 and \$ in 2017, all of which were recorded as long-term accrued liabilities as of December 31, 2016 and December 31, 2017, respectively, upon determination that the payment of such minimum royalties was probable and the amount was estimable. We are also obligated to pay to MSK certain clinical, regulatory and sales-based milestone payments under the MSK License, which payments become due upon achievement of the related clinical, regulatory or sales-based milestones. Certain of these clinical and regulatory milestone payments become due at the earlier of completion of the related milestone activity or the date indicated in the MSK License. Total potential clinical and regulatory milestones potentially due under the MSK License are \$2,450,000 and \$9,000,000, respectively. There are also sales-based milestones that become due should we achieve certain amounts of sales of licensed products resulting from the license arrangement with MSK, with total potential sales-based milestones potentially due of \$20,000,000. We have not entered into any sublicenses related to the MSK License. As product candidates progress through clinical development, regulatory approval and commercialization, certain milestone payments will come due either as a result of the milestones having been met or the passage of time even if the milestones have not been met. We will also owe MSK mid to high single digit royalties on commercial sales of our approved products, including an annual fixed minimum royalty of \$80,000 over the royalty term starting in 2020 whether or not product sales are ever achieved. In addition, to the extent we enter into sublicense arrangements, we are required to pay to MSK a percentage of certain payments that we receive from sublicensees of the rights licensed to us by MSK, which percentage will be based upon the date we receive such payments or the achievement of certain clinical milestones. Additionally, the terms of our MSK License and MSK CD33 License provide that MSK is entitled to receive 40-50% of any income generated from the sale of any PRV or the sale of any other comparable incentives provided by any non-U.S. jurisdiction that we may receive.

The MSK License will expire, on a country-by-country basis, and on a licensed-product-by-licensed-product or licensed-service-by-licensed-service basis, on the later of (i) the expiration of the last to expire of the patents and patent applications covering such licensed product or service in such country, (ii) the expiration of any market exclusivity period granted by a regulatory authority for such licensed product or service in such country, or (iii) 15 years from the first commercial sale of such licensed product or service in such country.

MSK may terminate the MSK License upon prior written notice in the event of our uncured material breach, or upon prior written notice if such breach is of a payment obligation. MSK may also terminate the MSK License upon written notice in the event of our bankruptcy or insolvency or our conviction of a felony relating to the licensed products, or if we challenge the validity or enforceability of any licensed patent right. In addition, we have the right to terminate the MSK License in its entirety at will upon prior written notice to MSK, but if we have commenced the commercialization of licensed products and/or licensed services we can only terminate at will if we cease all development and commercialization of such licensed products and/or licensed services.

In connection with these arrangements, on August 20, 2015 we also entered into a letter agreement with MSK pursuant to which we issued to MSK 1,428,500 shares of our common stock and agreed that if in the future we issued any shares of its capital stock, we would issue sufficient shares of common stock to MSK such that at all times prior to us obtaining equity financing equal to or greater

than \$25,000,000 in the aggregate, MSK shall hold shares of our common stock equal to 12.5% of the issued and outstanding shares of common stock (assuming full conversion or exercise of all outstanding preferred stock and other convertible securities, rights, options and warrants). Following issuances of our common stock in 2016, we issued to MSK an additional 479,328 on May 20, 2016 and 520,601 shares on August 20, 2016 in order for MSK to maintain the 12.5% ownership interest. As of December 31, 2016, MSK no longer has the right to receive additional shares of our common stock under the MSK License. Our failure to meet certain conditions under the MSK License could cause the related license to such licensed product to be canceled and could result in termination of the MSK License by MSK.

On November 10, 2015, we entered into the Sponsored Research Agreement, or the SRA, with MSK pursuant to which we committed to provide aggregate research funding to MSK of certain amounts annually for a term of five years. The research will be conducted in accordance with a written plan and budget approved by the parties. We have been granted a non-exclusive, non-commercial, non-transferable, royalty-free license to use any inventions or discoveries developed by MSK personnel that is within the scope of the information resulting from the project, for our internal, non-commercial research purposes. We have also been granted a first option to negotiate an exclusive or non-exclusive commercial license to MSK's rights in inventions developed by MSK personnel and a first option to negotiate an exclusive license to MSK's rights in inventions jointly developed by MSK and our personnel. The term of the SRA shall continue until the activities set forth in each statement of work entered into under the SRA are completed. The SRA may be terminated by either party upon prior written notice. During 2016, we incurred research and development expenses of \$1,099,000 under the SRA.

On September 20, 2016, we entered into a Master Data Services Agreement, or the MDSA, with MSK pursuant to which we committed to make certain payments to MSK annually in exchange for certain services, including transfer of clinical data and databases, regulatory files and other know-how to us by employees at MSK who are specifically assigned to assist with such services to us. Either party may terminate the MDSA upon prior written notice in the event of an uncured material breach. During 2016, we incurred expenses of \$265,000 under the MDSA.

Also, on June 21, 2017, we entered into the Investigator-Sponsored Master Clinical Trial Agreement, or the MCTA, as later amended on October 11, 2017, with MSK pursuant to which we committed to provide aggregate funding to MSK up to a certain amount for clinical studies to be conducted at MSK. Each such clinical study will be conducted in accordance with a written plan and budget and protocol approved by the parties. Under the MCTA, we and MSK have granted each other a non-exclusive, non-transferable, worldwide, royalty-free license, without right to sublicense, to use any inventions or discoveries developed by personnel of each such party, that is within the scope of the information resulting from the relevant study, for the other party's internal, non-commercial research purposes until such Invention is commercially available. We have also been granted a first option to negotiate an exclusive or non-exclusive commercial license to MSK's rights in inventions or discoveries developed by MSK personnel under this MCTA and a first option to negotiate an exclusive license to MSK's rights in inventions or discoveries jointly developed by MSK and our personnel under this MCTA. The MCTA will continue in effect through completion of the studies, and may be terminated by either party upon prior written notice.

On June 27, 2017, we entered into two separate Core Facility Service Agreements, or CFSAs, with MSK pursuant to which we committed to make certain payments to MSK in exchange for certain laboratory services over the term of the CFSAs. Either party may terminate either of these CFSAs for any reason, or for no reason, upon prior written notice. In the event of termination of either of these CFSAs, we will make full payment to MSK for all work performed on, or expenses related to, the project up to the date of termination including all non-cancelable obligations following receipt from MSK of any completed or in-process deliverables in connection with the project.

On November 13, 2017, we entered into a license agreement, or the MSK CD33 License, with MSK, which grants us a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics in connection with certain CD33 antibodies generated in a specific principal investigator's laboratory at MSK and constructs thereof. The MSK CD33 License is exclusive with respect to such patent rights and tangible materials within such know-how, and nonexclusive with respect to MSK's rights in such know-how and related intellectual property rights. As product candidates progress through clinical development, regulatory approval and commercialization, certain milestone payments will come due either as a result of the milestones having been met or the passage of time even if the milestones have not been met. Also, we will owe MSK customary royalties on commercial sales of our approved products, if any, including a fixed minimum royalty whether or not product sales are ever achieved. Total potential milestones due under the MSK CD33 License are \$550,000, \$500,000 and \$7,500,000 for clinical, regulatory and sales based milestones, respectively. In addition, the MSK CD33 License contains minimum royalty payments that become due beginning in year 10 of \$40,000 per year prior, subject to increase and creditable against any royalty payments due based on sales in the future. We are required to pay mid to high single digit royalties on sales of licensed products. We also agreed to pay MSK approximately \$1,360,000 for research services related to the intellectual property licensed under the MSK CD33 License. The research services are expected to occur over the two year period immediately following the date of the MSK CD33 License.

## **Government Regulation**

The FDA and other regulatory authorities at federal, state, and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring, and post-approval reporting of biologics such as those we are developing. We, along with third-party contractors, will be required to navigate the various pre-clinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources.

The process required by the FDA before biologic product candidates may be marketed in the United States generally involves the following:

- completion of pre-clinical laboratory tests and animal studies performed in accordance with the FDA's cGMP regulation;
- submission to the FDA of an IND, which must become effective before clinical trials may begin and must be updated annually or when significant changes are made;
- approval by an independent Institutional Review Board, or IRB, or ethics committee at each clinical site before the trial is begun;
- performance of adequate and well-controlled human clinical trials to establish the safety, purity and potency of the proposed biologic product candidate for its intended purpose;
- preparation of and submission to the FDA of a BLA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA Advisory Committee review, if applicable;

- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMP and to assure that the facilities, methods and controls are adequate to preserve the biological product's continued safety, purity and potency, and of selected clinical investigations to assess compliance with current Good Clinical Practices, or cGCPs; and
- FDA review and approval of the BLA to permit commercial marketing of the product for particular indications for use in the United States, which must be updated annually when significant changes are made.

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

A clinical trial involves the administration of the investigational product to human patients under the supervision of qualified investigators in accordance with cGCPs, which includes the requirement that all research patients provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the patients are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for patients or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- *Phase 1*—The investigational product is initially introduced into healthy human patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.

- *Phase 2*—The investigational product is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- *Phase 3*—The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA.

Phase 1, Phase 2 and Phase 3 testing may not be completed successfully within a specified period, if at all, and there can be no assurance that the data collected will support FDA approval or licensure of the product. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and potency. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

#### ***BLA Submission and Review by the FDA***

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, non-clinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from pertinent pre-clinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of the product, or from a number of alternative sources, including studies initiated by investigators. The submission of a BLA requires payment of a substantial User Fee to FDA, and the sponsor of an approved BLA is also subject to annual program fees. These fees are typically increased annually. A waiver of user fees may be obtained under certain limited circumstances.

Once a BLA has been submitted, the FDA's goal is to review the application within 10 months after it accepts the application for filing, or, if the application relates to an unmet medical need in a serious or life-threatening indication, six months after the FDA accepts the application for filing. The review process is often significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed, or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may convene an advisory committee to provide clinical insight on application review questions. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of

the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with cGCP. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The testing and approval process requires substantial time, effort and financial resources, and each may take several years to complete. The FDA may not grant approval on a timely basis, or at all, and we may encounter difficulties or unanticipated costs in our efforts to secure necessary governmental approvals, which could delay or preclude us from marketing our products. After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the product will be produced, the FDA may issue an approval letter, or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter may request additional information or clarification. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing regulatory standards is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies. In addition, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

A sponsor may seek approval of its product candidate under programs designed to accelerate FDA's review and approval of new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for fast track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address an unmet medical need for the condition. For a fast track product, the FDA may consider sections of the BLA for review on a rolling basis before the complete application is submitted if relevant criteria are met. A fast track designated product candidate may also qualify for priority review, under which the FDA sets the target date for FDA action on the BLA at six months after the FDA accepts the application for filing. Priority review is granted when there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of 10 months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval program, the FDA may approve a BLA on the basis of either a surrogate objective that is reasonably likely to predict clinical benefit, or on a clinical objective that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity,



rarity, or prevalence of the condition and the availability or lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval are generally required to verify the biologic's clinical benefit in relationship to the surrogate objective or ultimate outcome in relationship to the clinical benefit. In addition, a sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the product candidate is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant objectives, such as substantial treatment effects observed early in clinical development. Sponsors may request the FDA to designate a breakthrough therapy at the time of or any time after the submission of an IND, but ideally before an end-of-Phase 2 meeting with FDA. If the FDA designates a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the therapy; providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the non-clinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and considering alternative clinical trial designs when scientifically appropriate, which may result in smaller trials or more efficient trials that require less time to complete and may minimize the number of patients exposed to a potentially less efficacious treatment. Breakthrough designation also allows the sponsor to file sections of the BLA for review on a rolling basis.

Fast Track designation, priority review and BTB do not change the standards for approval but may expedite the development or approval process.

### ***Orphan Drugs***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, defined as a disease or condition with either a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 individuals in the United States when there is no reasonable expectation that the cost of developing and making available the drug or biologic in the United States will be recovered from sales in the United States for that drug or biologic. ODD must be requested before submitting a BLA. After the FDA grants ODD, the generic identify of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

If a product that has received ODD and subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of ODD are tax credits for certain research and a waiver of the BLA application user fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received ODD. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for

designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

### ***Rare Pediatric Disease Designation***

The Rare Pediatric Disease Priority Review Voucher Program, or the PRV Program, is intended to incentivize pharmaceutical companies to develop drugs for rare pediatric diseases. A company that obtains approval of an IND or a BLA for a designated rare pediatric disease may be eligible for a PRV from the FDA, which may be redeemed to obtain priority review for a subsequent new drug application or BLA by the owner of such PRV. A PRV is fully transferrable and can be sold to any company, who in turn can redeem the PRV for priority review of a marketing application in six months, compared to the standard timeframe of approximately ten months. In December 2016, the House of Representatives approved the 21st Century Cures Act, which among other initiatives reauthorizes the PRV Program for rare pediatric diseases until 2020. A drug that receives a RPDD before October 1, 2020 continues to be eligible for a PRV if the drug is approved before October 1, 2022.

### ***Post-Approval Requirements***

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance. We cannot be certain that we or our present or future suppliers will be able to comply with the cGMP regulations and other FDA regulatory requirements. If our present or future suppliers are not able to comply with these requirements, the FDA may, among other things, halt our clinical trials, require us to recall a product from distribution, or withdraw approval of the BLA.

We rely, and expect to continue to rely, on third parties for the production of clinical quantities of our product candidates, and expect to rely in the future on third parties for the production of commercial quantities. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved BLA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing. The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or

frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

### ***Pediatric Studies and Exclusivity***

Under the Pediatric Research Equity Act of 2003, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time. For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of an applicant, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

The FDA Reauthorization Act of 2017 established new requirements to govern certain molecularly targeted cancer indications. Any company that submits a BLA three years after the date of enactment of that statute must submit pediatric assessments with the BLA if the biologic is intended for the treatment of an adult cancer and is directed at a molecular target that FDA determines to be substantially relevant to the growth or progression of a pediatric cancer. The investigation must be designed to yield clinically meaningful pediatric study data regarding the dosing, safety and preliminary efficacy to inform pediatric labeling for the product.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

### ***Other Healthcare Laws and Compliance Requirements***

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of drug products that are granted regulatory approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain our business and/or financial arrangements. Such restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration (including any kickback, bribe or rebate), directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, lease or order of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willingly executing, or attempting to execute, a scheme or making false statements in connection with the delivery of or payment for health care benefits, items, or services;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information on covered entities and their business associates that associates that perform certain functions or activities that involve the use or disclosure of protected health information on their behalf;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or collectively the ACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the U.S. Department of Health and Human Services, information related to payments and other

transfers of value to physicians and teaching hospitals and information regarding ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Also, the U.S. Foreign Corrupt Practices Act and similar worldwide anti-bribery laws generally prohibit companies and their intermediaries from making improper payments to foreign officials for the purpose of obtaining or retaining business. We cannot assure you that our internal control policies and procedures will protect us from reckless or negligent acts committed by our employees, future distributors, partners, collaborators or agents. Violations of these laws, or allegations of such violations, could result in fines, penalties or prosecution and have a negative impact on our business, results of operations and reputation.

### ***Coverage and Reimbursement***

Sales of pharmaceutical products depend significantly on the availability of third-party coverage and reimbursement. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. Although we currently believe that third-party payors will provide coverage and reimbursement for our product candidates, if approved, these third-party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. In addition, significant uncertainty exists as to the reimbursement status of newly approved healthcare products. We may need to conduct expensive clinical studies to demonstrate the comparative cost-effectiveness of our products. The product candidates that we develop may not be considered cost-effective. It is time consuming and expensive for us to seek coverage and reimbursement from third-party payors. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement may not be available or sufficient to allow us to sell our products on a competitive and profitable basis.

### ***Review and Approval of Medicinal Products in the European Union***

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, an applicant will need to obtain the necessary approvals by the comparable non-U.S. regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. Specifically, the process governing approval of medicinal products in the European Union generally follows the same lines as in the United States. It entails satisfactory completion of pre-clinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing

authorization by these authorities before the product can be marketed and sold in the European Union.

The Clinical Trials Directive 2001/20/EC, the Directive 2005/28/EC on Good Clinical Practice, or GCP, and the related national implementing provisions of the individual EU member states, or EU Member States govern the system for the approval of clinical trials in the European Union. Under this system, an applicant must obtain prior approval from the competent national authority of the EU Member States in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial at a specific study site after the competent ethics committee has issued a favorable opinion. The clinical trial application must be accompanied by, among other documents, an investigational medicinal product dossier, or the Common Technical Document, with supporting information prescribed by Directive 2001/20/EC, Directive 2005/28/EC, where relevant the implementing national provisions of the individual EU Member States and further detailed in applicable guidance documents.

In April 2014, the new Clinical Trials Regulation, (EU) No 536/2014, or the Clinical Trials Regulation, was adopted, and is anticipated to enter into force in 2019. The Clinical Trials Regulation will be directly applicable in all the EU Member States, repealing the current Clinical Trials Directive 2001/20/EC. Conduct of all clinical trials performed in the European Union will continue to be bound by currently applicable provisions until the new Clinical Trials Regulation becomes applicable. The extent to which on-going clinical trials will be governed by the Clinical Trials Regulation will depend on when the Clinical Trials Regulation becomes applicable and on the duration of the individual clinical trial. If a clinical trial continues for more than three years from the day on which the Clinical Trials Regulation becomes applicable, the Clinical Trials Regulation will at that time begin to apply to the clinical trial.

The Clinical Trials Regulation aims to simplify and streamline the approval of clinical trials in the European Union. The main characteristics of the regulation include: a streamlined application procedure via a single entry point, the "EU portal"; a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (EU Member States concerned). Part II is assessed separately by each EU Member State concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the Clinical Trials Regulation.

To obtain a marketing authorization for a product under European Union regulatory systems, an applicant must submit an MAA either under a centralized procedure administered by the EMA, or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the European Union. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the European Union, applicants have to demonstrate compliance with all measures included in an EMA-approved Paediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver or (3) a deferral for one or more of the measures included in the PIP.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all EU Member States and three of the four European Free Trade Association, or EFTA, States, Iceland, Liechtenstein and Norway. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal

products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases, including products for the treatment of cancer. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional.

Under the centralized procedure, the Committee for Medicinal Products for Human Use, or the CHMP, established at the EMA is responsible for conducting the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. If the CHMP accepts such request, the time limit of 210 days will be reduced to 150 days but it is possible that the CHMP can revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment. At the end of this period, the CHMP provides a scientific opinion on whether or not a marketing authorization should be granted in relation to a medicinal product. Within 15 calendar days of receipt of a final opinion from the CHMP, the European Commission must prepare a draft decision concerning an application for marketing authorization. This draft decision must take the opinion and any relevant provisions of EU law into account. Before arriving at a final decision on an application for centralized authorization of a medicinal product the European Commission must consult the Standing Committee on Medicinal Products for Human Use. The Standing Committee is composed of representatives of the EU Member States and chaired by a non-voting European Commission representative. The European Parliament also has a related "droit de regard". The European Parliament's role is to ensure that the European Commission has not exceeded its powers in deciding to grant or refuse to grant a marketing authorization.

Unlike the centralized authorization procedure, the decentralized marketing authorization procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States who, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States.

The mutual recognition procedure similarly is based on the acceptance by the competent authorities of the EU Member States of the marketing authorization of a medicinal product by the competent authorities of other EU Member States. The holder of a national marketing authorization may submit an application to the competent authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the competent authority of another EU Member State.

In the European Union, innovative medicinal products approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity pursuant to Directive 2001/83/EC. Regulation (EC) No 726/2004 repeats this entitlement for medicinal products authorized in accordance the centralized authorization procedure. Data exclusivity prevents applicants for authorization of generics of these innovative products from referencing the innovator's data to assess a generic (abbreviated) application

for a period of eight years. During an additional two-year period of market exclusivity, a generic marketing authorization application can be submitted and authorized, and the innovator's data may be referenced, but no generic medicinal product can be placed on the European Union market until the expiration of the market exclusivity. The overall 10-year period will be extended to a maximum of 11 years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity so that the innovator gains the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, pre-clinical tests and clinical trials.

A marketing authorization has an initial validity for five years in principle. The marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the EU Member State. To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide, on justified grounds relating to pharmacovigilance, to proceed with one further five year period of marketing authorization. Once subsequently definitively renewed, the marketing authorization shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the European Union market (in case of centralized procedure) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a drug can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union when the application is made, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that the marketing of the drug in the European Union would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Once authorized, orphan medicinal products are entitled to 10 years of market exclusivity in all EU Member States and in addition a range of other benefits during the development and regulatory review process including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure covering all member countries and a reduction or elimination of registration and marketing authorization fees. However, marketing authorization may be granted to a similar medicinal product with the same orphan indication during the 10 year period with the consent of the marketing authorization holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity.



In case an authorization for a medicinal product in the European Union is obtained, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include:

- Compliance with the European Union's stringent pharmacovigilance or safety reporting rules must be ensured. These rules can impose post-authorization studies and additional monitoring obligations.
- The manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory, must also be conducted in strict compliance with the applicable European Union laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice. These requirements include compliance with European Union cGMP standards when manufacturing medicinal products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the European Union with the intention to import the active pharmaceutical ingredients into the European Union.
- The marketing and promotion of authorized drugs, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the European Union notably under Directive 2001/83EC, as amended, and EU Member State laws.

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union (commonly referred to as "Brexit"). Thereafter, on March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Treaty of Lisbon Amending the Treaty on European Union and the Treaty Establishing the European Community. The withdrawal of the United Kingdom from the European Union will take effect either on the effective date of the withdrawal agreement or, in the absence of agreement, two years after the United Kingdom provides a notice of withdrawal pursuant to the Treaty on European Union. Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from EU directives and regulations, Brexit could materially impact the future regulatory regime which applies to products and the approval of product candidates in the United Kingdom. It remains to be seen how, if at all, Brexit will impact regulatory requirements for product candidates and products in the United Kingdom.

### ***Healthcare Reform***

A primary trend in the United States healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.

In March 2010, the U.S. Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for drug products under government health care programs. Among the provisions of the ACA of importance to our potential product candidates are:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the

federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to two percent (2%) per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken.

Since its enactment, there have been numerous legal challenges and Congressional actions to repeal and replace provisions of the ACA. Some of the provisions of the ACA have yet to be implemented, and there have been legal and political challenges to certain aspects of the ACA. Since January 2017, President Trump has signed two executive orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the ACA. Moreover, the Tax Reform Bill was enacted on December 22, 2017, and includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Congress may consider other legislation to repeal or replace additional elements of the ACA. We continue to evaluate the effect that the ACA, the repeal of the individual mandate, and any additional repeal and replacement efforts may have on our business but expect that the ACA, as currently enacted or as it may be amended in the future, and other healthcare reform measures that may be adopted in the future could have a material adverse effect on our industry generally and on our ability to maintain or increase sales of our existing products that we successfully commercialize or to successfully commercialize our product candidates, if approved. In addition to the ACA, there will continue to be proposals by legislators at both the federal and state levels, regulators and third party payors to keep healthcare costs down while expanding individual healthcare benefits.

## **Employees**

As of December 31, 2017, we had 12 full time employees. The members of our management team are employed by both our company and Y-mAbs Therapeutics A/S, our wholly owned Danish subsidiary. As our development and commercialization plans and strategies develop, we intend to continue adding a number of additional managerial, operational, sales, marketing, financial, and other personnel. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

## **Facilities**

Our corporate headquarters are located in New York, New York, where we currently lease less than 1,000 square feet of office space pursuant to a lease renewal agreement expiring in May 2018, which is automatically renewed for successive periods equal to the current term but no less than three months unless terminated by either party upon at least three months' notice to the other. In March 2018 or soon thereafter, we expect to begin the process of transitioning to a new office space where we have leased 4,312 square feet pursuant to a new lease agreement dated as of January 10, 2018, which expires five years from the date we first begin to occupy the premises.

Our wholly owned Danish subsidiary, Y-mAbs Therapeutics A/S, leases approximately 1450 square feet of office space in Rungsted Kyst, Denmark pursuant to a lease agreement with one of

our largest shareholders dated as of May 30, 2016, which can be terminated by either party upon three months' notice. On April 1, 2018, we expect to begin the process of transitioning to a new office space where we have leased 7,373 square feet pursuant to a new lease agreement dated February 2, 2018, which expires on March 1, 2021 and may subsequently be cancelled by us with six months notice by September 2021. The landlord may not terminate the lease until March 1, 2024.

We believe that suitable additional or alternative space for either location would be available as required in the future on commercially reasonable terms.

We believe that suitable additional or alternative space for both our U.S. and Danish locations would be available as required in the future on commercially reasonable terms.

### **Legal Proceedings**

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently subject to any legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have an adverse effect on our business, operating results or financial condition.

**MANAGEMENT****Executive Officers and Directors**

Our executive officers and directors, and their ages and positions as of December 31, 2017 are as set forth below:

<b>Name</b>	<b>Age</b>	<b>Position</b>
<b>Executive Officers</b>		
Thomas Gad(3)	47	Founder, Chairman of the Board of Directors, President, Head of Business Development
Claus Juan Møller San Pedro, M.D., Ph.D.	55	Chief Executive Officer and Director
Bo Kruse	45	Executive Vice President, Secretary, Treasurer, Chief Financial Officer and Director
Torben Lund-Hansen, Ph.D.	67	Senior Vice President and Head of Technical Operations
Steen Lisby, M.D., DMSc	54	Senior Vice President and Chief Medical Officer
Joris Wiel Jan Wilms	44	Senior Vice President and Chief Operating Officer
<b>Non-Employee Directors</b>		
Johan Wedell-Wedellsborg(2)	48	Director
Gregory Raskin, M.D.(1)(3)	45	Director
Michael Buschle, Ph.D.(2)(3)	57	Director
James I. Healy, M.D.(2)	53	Director
Ashutosh Tyagi, M.D.(1)	41	Director
David N. Gill(1)	63	Director

(1) Member of audit committee.

(2) Member of compensation committee.

(3) Member of nominating and corporate governance committee.

**Executive Officers**

**Thomas Gad** founded our company in April 2015 and has served as our Chairman, President and Head of Business Development and Strategy and as a member of our board of directors since our inception. Mr. Gad founded our company inspired by his daughter, who went through six years of various cancer treatments before receiving breakthrough cancer immunotherapy at MSK and overcoming high-risk NB. He was also responsible for securing executive management and seed capital for our company. Mr. Gad has more than 12 years of industry experience in the pharmaceutical industry, including business development, senior management, financing and licensing negotiations and manufacturing site qualification. Mr. Gad was the founder and sole owner of Y-mAbs Holding, ApS, a personal holding company involved in research and development activities in the pharmaceutical industry from 2014 until the company was placed in liquidation proceedings in 2015. This company is unrelated to our company. He was the co-founder of Singad Pharma, a Danish specialty pharmaceutical and distribution company, from 2003 to 2013. Prior to that, Mr. Gad worked with Aspen Capital Partners/FFC A/S in investment banking from 1998 to 2003 and has extensive experience in raising capital for publicly listed companies. Mr. Gad has a Bachelor of Science in Business Administration from Pepperdine University.

**Claus Juan Møller San Pedro, M.D., Ph.D.** has served as our Chief Executive Officer since June 2015. Dr. Møller was the founder of Azanta A/S, or Azanta, a Danish specialty biopharmaceutical company and its Chief Executive Officer from 2009 to 2015. In addition, Dr. Møller was a co-founder of Genmab A/S, or Genmab, one of the largest European biopharmaceutical companies. Dr. Møller joined Genmab in 1999, where he served as Executive Vice President and Chief Operating Officer until 2008. Dr. Møller has also held previous executive management positions at various biopharmaceutical companies, including Executive Vice President, Chief Medical and Chief Operating Officer of OXiGENE, Inc., and Medical Director of Synthélabo Scandinavia. Dr. Møller received his M.D. and Ph.D. degrees from the University of Copenhagen.

**Bo Kruse** has served as our Executive Vice President, Secretary, Treasurer and Chief Financial Officer since June 2015. Mr. Kruse has broad international finance experience, including knowledge of capital markets, accounting and other financing activities. Prior to joining our company, Mr. Kruse was Azanta's Chief Financial Officer from 2009 to 2015. Further, Mr. Kruse served as Genmab's Vice President and Chief Financial Officer from 2005 to 2008 and Vice President and Chief Accounting Officer from 2000 to 2005. During his tenure at Genmab, Mr. Kruse was directly involved in several financing rounds, including Genmab's initial public offering in 2000. Mr. Kruse has a Master's of Science in Business Economics and Auditing from the Copenhagen Business School.

**Torben Lund-Hansen, Ph.D.** has served as our Senior Vice President, Head of Technical Operations since January 2016. Dr. Lund-Hansen has substantial experience in antibody process development, commercial manufacturing and global project management. Dr. Lund-Hansen was Vice President and Head of Manufacturing from 2002 to 2006, Vice President and Head of Manufacturing and Preclinical Safety from 2006 to 2008, Senior Vice President, Technical Operations from 2008 to 2009 at Genmab and President and Treasurer at Genmab MN Inc. from 2008 to 2009. At Genmab, Dr. Lund-Hansen was responsible for outsourcing of clinical and commercial drug substance and drug product manufacturing. He was also President and Treasurer from 2008 to 2009 of Genmab MN Inc., a wholly owned subsidiary of Genmab located near Minneapolis-St. Paul, Minnesota. Dr. Lund-Hansen has been responsible for compiling technical Chemistry, Manufacturing, and Controls, or CMC, documentation packages submitted to global regulatory agencies followed by approval and launch of several biologics. Dr. Lund-Hansen received his M.Sc. and Ph.D. from the University of Copenhagen.

**Steen Lisby, M.D., DMSc** joined our company in June 2017 as our Senior Vice President and Chief Medical Officer. Dr. Lisby has extensive clinical and scientific experience, and is the author of over 50 scientific peer-reviewed publications in clinical research. Previously, Dr. Lisby was Vice President, Head of Medical at Genmab A/S from 2014 to 2017 and also held other positions there including Senior Medical Director from 2010 to 2014, Medical Director from 2008 to 2010 and Medical Advisor from 2004 to 2007. Dr. Lisby received his M.D. degree from the University of Copenhagen and is a named inventor on seven patent applications.

**Joris Wiel Jan Wilms** has served as our Senior Vice President and Chief Operating Officer since November 2017. Mr. Wilms joined our company in July 2016 as Vice President and Head of Clinical Operations and has extensive industry experience in clinical development, primarily within oncology and hematology indications. Mr. Wilms was at KLIFO A/S, or KLIFO, from 2010 to 2016, where he served as Vice President—Clinical Trial Services and Pharmacovigilance Services, and at Genmab from 2004 to 2010, where he served as Associate Director of Clinical Development from 2008 to 2010. At KLIFO and Genmab, he was responsible for overseeing several first-in-human studies and pivotal clinical trials, leading to the approval of two monoclonal antibody-based products. Mr. Wilms received his M.Sc. in Pharmacy from the University of Groningen in The Netherlands.

## Non-Employee Directors

Information regarding the members of our Board of Directors who are not also executive officers is set forth below:

Johan Wedell-Wedellsborg has been a member of our Board of Directors since September 2015. Mr. Wedell-Wedellsborg has been the owner and Chairman of the Board of Weco Group A/S, or Weco, one of our principal stockholders, since May 2001. Weco is involved in shipping, investments in biotechnology companies, real estate investments and the financial services industry. Mr. Wedell-Wedellsborg is also the majority owner of WG Biotech ApS, another one of our principal stockholders. We believe that Mr. Wedell-Wedellsborg is qualified to serve on our Board of Directors due to his educational background, his extensive business experience and his experience in investing in the biotechnology and life sciences industry. Mr. Wedell-Wedellsborg is a member of our Compensation Committee.

Gregory Raskin, M.D. has been a member of our Board of Directors since September 2015. Dr. Raskin is Vice President, Technology Development at Memorial Sloan Kettering Cancer Center, where he has worked since 2012. Dr. Raskin holds a B.A. in Molecular Biophysics and Biochemistry and an M.D. from Yale University. We believe that Dr. Raskin is qualified to serve on our Board of Directors due to his educational background and extensive experience in working in the biotechnology and life sciences industry. Dr. Raskin is a member of our Audit Committee and our Nominating and Corporate Governance Committee.

Michael Buschle, Ph.D. has been a member of our Board of Directors since October 2017, representing HBM Healthcare Investments (Cayman) Ltd., or HBM, one of our principal stockholders. Dr. Buschle has over 25 years of experience in the biotechnology and pharmaceutical industry and related research. Since June 2017, Dr. Buschle has been a consultant and venture partner of HBM Partners AG, a private equity company that focuses on biopharmaceutical and other healthcare-related companies in Europe, North America, India and other emerging markets. From April 2006 to December 2016, Dr. Buschle held various positions of increasing seniority with Glenmark Pharmaceuticals Ltd., or Glenmark, including President Biologics and Chief Scientific Officer. Prior to Glenmark, Dr. Buschle held various positions at Intercell AG, or Intercell, a biopharmaceutical company of which he was one of the Co-Founders, including Chief Scientific Officer. Prior to forming Intercell, Dr. Buschle held a position at the pharmaceutical company Boehringer Ingelheim GmbH, Vienna. Dr. Buschle's scientific career has included work at the Royal Free Hospital School of Medicine, London, United Kingdom, the St. Jude Children's Research Hospital, Memphis, Tennessee and at the Boehringer Ingelheim-owned Institute of Molecular Pathology, Vienna, Austria. Dr. Buschle holds a Doctorate from the University of London and is the holder of several patents in the field of biotechnology. We believe Dr. Buschle is qualified to serve on our Board of Directors due to his educational background and extensive experience in investing and working in the biotechnology and life sciences industry, as well as his prior service as a senior-level executive in a number of pharmaceutical and biotechnology communities. Dr. Buschle is a member of our Compensation Committee and our Nominating and Corporate Governance Committee.

James I. Healy, M.D., Ph.D. has served as a member of our board of directors since November 2017. Dr. Healy has been a general partner at Sofinnova Ventures, a venture capital firm, since 2000. Prior to Sofinnova Ventures, Dr. Healy held various positions at Sanderling Ventures, a venture capital firm, Bayer Healthcare Pharmaceuticals (as successor to Miles Laboratories), a research based pharmaceutical company and ISTA Pharmaceuticals, Inc., a company specializing in ophthalmic pharmaceutical products. Dr. Healy is currently on the board of directors of Ascendis Pharma A/S, Coherus BioSciences, Inc., Edge Therapeutics, Inc., Obseva SA, Natera, Inc., NuCana plc and several private companies. Previously, Dr. Healy served as a board member of Auris Medical Holding AG, Anthera Pharmaceuticals, Inc., Amarin Corporation plc, Durata Therapeutics, Inc., InterMune, Inc.,

KaloBios Pharmaceuticals, Inc., Hyperion Therapeutics, Inc., and a number of private companies. Dr. Healy holds a Bachelor of Arts in Molecular Biology and Scandinavian Studies from the University of California at Berkeley, and an M.D. and Ph.D. in Immunology from Stanford University School of Medicine. Our board of directors believes that Dr. Healy is qualified to serve as a director due to his significant medical background, extensive experience investing and working in the life science industry, and his extensive service on the boards of directors of other public and private life sciences companies. Dr. Healy currently serves as the chair of our compensation committee.

Ashutosh Tyagi, M.D. has been a member of our Board of Directors since November 2017, representing Scopia Capital Management LP, or Scopia Capital, an institutional alternative asset management firm with over \$6 billion of assets under management. Scopia Capital is the investment manager of two of our major stockholders. Dr. Tyagi has been with Scopia Capital since 2010 and a partner since 2012. At Scopia Capital, Dr. Tyagi manages global health care investments and is a Co-Portfolio Manager of Scopia Capital's health care funds. Dr. Tyagi received a B.A. in Asian Studies from the University of Michigan, an MBA from the University of Michigan Business School and an M.D. from the University of Michigan School of Medicine. We believe that Dr. Tyagi is qualified to serve on our Board of Directors due to his educational background and his extensive experience in investing and working in the biotechnology and life sciences industry. Dr. Tyagi is a member of our Audit Committee.

David N. Gill has been a member of our Board of Directors since December 2017. Mr. Gill was Chief Financial Officer of EndoChoice Holdings, Inc., a publicly traded medical device company, from August 2014 until it was sold to Boston Scientific Corporation in November 2016, and served as President and Chief Operating Officer of EndoChoice Holdings, Inc. from March 2016 to November 2016. He was the Chief Financial Officer of INC Research Holdings Inc., a clinical research organization, from February 2011 to August 2013, and served as a board member and audit committee chairman of INC Research Holdings Inc. from 2007 to 2010. From March 2009 to February 2011, Mr. Gill was the Chief Financial Officer of TransEnterix, Inc., a then private medical device company. Mr. Gill was Chief Financial Officer and Treasurer of NxStage Medical, Inc., a publicly traded dialysis equipment company, from July 2005 to November 2006. Mr. Gill served as Senior Vice President and Chief Financial Officer of CTI Molecular Imaging, Inc., a publicly traded medical imaging company, from January 2002 to May 2005, until its sale to Siemens AG. Since February 2015, he has served as a director and chair of the audit committee of Histogenics Inc., a publicly traded cellular therapy company. Mr. Gill has also served as a director and chair of the audit committee of Melinta Therapeutics, Inc. (formerly Cemptra, Inc.), a publicly traded pharmaceutical company focused on infectious disease, since April 2012. From 2006 to 2011, he served on several public and private company boards of directors, including those of LeMaitre Vascular, a publicly traded medical device company, and IsoTis, Inc., a publicly traded orthobiologics company that was acquired by Integra LifeSciences Holdings Corporation in October 2007. Mr. Gill holds a B.S. degree, cum laude, in Accountancy from Wake Forest University and an MBA with honors from Emory University. Mr. Gill was formerly a certified public accountant. We believe that Mr. Gill is qualified to serve as a director due to his education and experience in accounting and finance, his extensive experience as an executive in the biotechnology industry, his prior service as a senior-level executive in mature biotechnology companies and his service as a director of various publicly traded companies. Mr. Gill serves as Chair of our Audit Committee.

#### **Board Composition and Election of Directors**

As of the date hereof, our board of directors consists of nine members. Our directors hold office until their successors have been elected and qualified, or until the earlier of their resignation, removal or death.

The members of our board of directors were elected in compliance with certain voting provisions contained in a stockholders agreement among us and our stockholders. The stockholders agreement will terminate by its terms upon the completion of this offering and we will have no further contractual obligations regarding the election of our directors. See "Certain Relationships and Related Party Transactions." Our directors hold office until their successors have been elected and qualified or until their earlier death, resignation or removal. There are no family relationships among any of our directors or executive officers.

### ***Staggered Board***

Our amended and restated certificate of incorporation will provide that our board of directors will be divided into three classes of directors, with the classes as nearly equal in number as possible. Upon completion of this offering, each of these classes will be comprised of the following directors:

- Our Class I directors will be \_\_\_\_\_ ;
- Our Class II directors will be \_\_\_\_\_ ; and
- Our Class III directors will be \_\_\_\_\_ .

Subject to any earlier resignation or removal in accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws that we expect to be in effect upon the closing of this offering, our Class I directors will serve until the first annual meeting of stockholders following the completion of this offering; our Class II directors will serve until the second annual meeting of stockholders following the completion of this offering; and our Class III directors will serve until the third annual meeting of stockholders following the completion of this offering.

Our amended and restated certificate of incorporation will provide that the number of our directors shall be fixed from time to time by a resolution of our board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class shall consist of one third of the board of directors.

### **Director Independence**

Applicable Nasdaq Stock Market, or Nasdaq, rules require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act, and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under applicable Nasdaq rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: the source of compensation of the director, including any



consulting, advisory or other compensatory fee paid by such company to the director; and whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In 2018, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of Dr. Healy, Dr. Buschle, Dr. Tyagi and Mr. Gill is an "independent director" as defined under applicable Ndaq rules, including, in the case of all the members of our audit committee, the independence criteria set forth in Rule 10A-3 under the Exchange Act, and in the case of all the members of our compensation committee, the independence criteria set forth in Rule 10C-1 under the Exchange Act. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director. Mr. Gad, Dr. Møller and Mr. Kruse are not independent directors under these rules because they are executive officers of the company and Mr. Wedell-Wedellsborg and Dr. Raskin are not independent directors because they are affiliates of two of our principal shareholders, WG Biotech ApS, or WG Biotech, and MSK, respectively.

### **Board Committees**

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee. Each of these committees will operate under a charter that has been approved by our board of directors. The composition of each committee will be effective as of the date of this prospectus.

#### ***Audit Committee***

The members of our audit committee are Mr. Gill, Dr. Raskin and Dr. Tyagi. Mr. Gill is the chair of the audit committee. Effective as of the date of this prospectus, our audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from that firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our internal audit function, if any;
- discussing our risk assessment and risk management policies;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting-related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management;
- reviewing and approving or ratifying any related person transactions; and

- preparing the audit committee report required by the SEC rules.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our board of directors has determined that Mr. Gill is an "audit committee financial expert" as defined in applicable SEC rules and that each of the members of our audit committee possesses the financial sophistication required for audit committee members under Nasdaq rules. We believe that the composition of our audit committee will meet the requirements for independence under current Nasdaq and SEC rules and regulations.

#### ***Compensation Committee***

The members of our compensation committee are Dr. Buschle, Dr. Healy and Mr. Wedell-Wedellsborg. Dr. Healy is the chair of the compensation committee. Effective as of the date of this prospectus, our compensation committee's responsibilities will include:

- reviewing and approving, or making recommendations to our board of directors with respect to, the compensation of our chief executive officer and our other executive officers;
- overseeing an evaluation of our senior executives;
- reviewing and making recommendations to our board of directors with respect to our incentive-compensation and equity-based compensation plans;
- overseeing and administering our equity-based plans;
- reviewing and making recommendations to our board of directors with respect to director compensation;
- reviewing and discussing annually with management our "Compensation Discussion and Analysis" disclosure if and to the extent then required by SEC rules; and
- preparing the compensation committee report if and to the extent then required by SEC rules.

We believe that the composition of our compensation committee will meet the requirements for independence under current Nasdaq and SEC rules and regulations.

#### ***Nominating and Corporate Governance Committee***

The members of our nominating and corporate governance committee are Mr. Gad, Dr. Buschle, and Dr. Raskin. Mr. Gad is the chair of the nominating and corporate governance committee. Effective as of the date of this prospectus, our nominating and corporate governance committee's responsibilities will include:

- identifying individuals qualified to become members of our board of directors;
- recommending to our board of directors the persons to be nominated for election as directors and to each of our board's committees;
- developing and recommending to our board of directors corporate governance principles; and
- overseeing an annual evaluation of our board of directors.

We believe that the composition of our nominating and corporate governance committee will meet the requirements for independence under current Nasdaq and SEC rules and regulations.

### **Compensation Committee Interlocks and Insider Participation**

None of our executive officers currently serves, or in the past year has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. None of the members of our compensation committee is, or has ever been, an officer or employee of our company.

### **Code of Business Conduct and Ethics**

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. Following this offering, we will post a copy of the code on the Corporate Governance section of our website. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

**EXECUTIVE AND DIRECTOR COMPENSATION**

This section describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers in 2017, and to each of our non-employee directors in 2017. We are an "emerging growth company" within the meaning of the JOBS Act, and have elected to comply with the reduced compensation disclosure requirements available to emerging growth companies under the JOBS Act. Our named executive officers for 2017 were Thomas Gad, Dr. Claus Juan Møller San Pedro, and Bo Kruse. This section also provides certain qualitative information regarding the manner and context in which compensation is awarded to and earned by our named executive officers and is intended to place in perspective the data presented in the tables and narrative that follow.

In preparing to become a public company, we have begun a thorough review of all elements of our executive compensation program, including the function and design of our equity incentive programs. We have begun, and we expect to continue in the coming months, to evaluate the need for revisions to our executive compensation program to ensure our program is competitive with the companies with which we compete for executive talent and is appropriate for a public company.

**Summary Compensation Table**

The following table shows the total compensation paid or accrued during the fiscal year ended December 31, 2017, to our Chief Executive Officer and our two next most highly compensated executive officers who earned more than \$100,000 during the fiscal year ended December 31, 2017, and were serving as executive officers as of such date. No option or other equity awards were granted to such executive officers during 2017.

<u>Name and Principal Position</u>		<u>Salary (\$)</u>	<u>Bonus (\$)(1)(2)</u>	<u>Option Awards (\$)</u>	<u>All Other Compensation (\$)(3)</u>	<u>Total (\$)(2)</u>
Thomas Gad <i>Founder, Chairman, President and Head of Business Development</i>	2017	\$ 350,004	\$	\$ 0	\$ 98,883	\$
Dr. Claus Juan Møller San Pedro M.D., Ph.D. <i>Chief Executive Officer</i>	2017	\$ 409,561(4)	\$	\$ 0	\$ 85,031	\$
Bo Kruse <i>Executive Vice President, Secretary, Treasurer and Chief Financial Officer</i>	2017	\$ 317,926	\$	\$ 0	\$ 1,031	\$

- (1) Except where noted otherwise, the amounts reported in the "Bonus" column represent discretionary annual cash bonuses awarded to our named executive officers. Our named executive officers have not received any non-cash compensation in lieu of salary or bonus.
- (2) The 2017 bonus amounts will be updated once the information is determined, which we anticipate will be in \_\_\_\_\_, 2018.
- (3) Each of Mr. Gad, Dr. Møller and Mr. Kruse serves as a member of our board of directors but do not receive any additional compensation for their service as a director. Amounts in this column include a monthly housing allowance of \$7,000 to cover rental expenses associated with the U.S. residence for each of Mr. Gad and Dr. Møller, as well as approximately \$14,000 of one time moving and storage expenses for Mr. Gad. Amounts also include certain insurance premiums and technology expenses paid for by us.
- (4) 60% of Dr. Møller's base salary is paid to him from our U.S. office and the remaining 40% is paid to him from our Danish office.

## Narrative to Summary Compensation Table

### **Base Salary**

In 2017, we paid annual base salaries of \$350,004 to Mr. Gad, \$409,561 to Dr. Møller, and \$317,926 to Mr. Kruse. 100% of Mr. Gad's base salary is paid to him from our U.S. office. 60% of Dr. Møller's base salary is paid to him from our U.S. office and the remaining 40% is paid to him from our Danish office. 100% of Mr. Kruse's base salary is paid to him from our Danish office.

We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our named executive officers. None of our named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary. For additional information regarding the employment agreements of our named executive officers, see the subsection entitled "—Employment Agreements".

### **Annual Bonus**

We do not have a formal performance-based bonus plan. From time to time, our board of directors has approved discretionary annual cash bonuses to our named executive officers with respect to their prior year performance. Mr. Gad, Dr. Møller and Mr. Kruse may receive cash bonuses for services performed during 2017. The Compensation Committee has not yet determined the amounts of such bonuses. We will disclose bonus amounts when they are determined. It is anticipated that such bonuses, if any, will be determined in \_\_\_\_\_, 2018. The annual incentive cash bonus has a target of 50% of the named executive officer's annual base salary and may be increased if our board of directors determines that the named executive officer has exceeded the performance objectives that year.

### **Equity Incentives**

Although we do not have a formal policy with respect to the grant of equity incentive awards to our named executive officers or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our named executive officers with a strong link to our long-term performance, create an ownership culture and help to align the interests of our named executive officers and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our named executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our named executive officers and from time to time may grant equity incentive awards to them in the form of stock options under our 2015 Plan, which may be granted as either incentive stock options or nonstatutory stock options.

Mr. Gad, Dr. Møller and Mr. Kruse did not receive any option or other equity awards during 2017, however, each of them may receive equity awards in 2018. The Compensation Committee has not yet determined the amounts of such equity awards, if any. We will disclose any such equity awards when they are determined. It is anticipated that such equity awards, if any, will be determined in \_\_\_\_\_, 2018.

### **Description of Option Awards**

On October 21, 2016, Mr. Gad, Dr. Møller and Mr. Kruse each received options to purchase 166,000, 200,000 and 133,000 shares, respectively, of our common stock at an exercise price of \$4.38 per share. The shares subject to each option will vest and become exercisable based on our "Standard Vesting Schedule" of 25% on the one-year anniversary of the date of grant, and 1/48<sup>th</sup> of the total shares subject to the option award vesting on the same day of the month as the grant date over the course of the next three years, subject to the executive's continued employment on each vesting date.

On December 14, 2016, Mr. Gad, Dr. Møller and Mr. Kruse each received options to purchase 16,000, 18,000 and 14,000 shares, respectively, of our common stock at an exercise price of \$8.50 per share, subject to our Standard Vesting Schedule.

In the event of a change of control, as defined in the 2015 Plan (as summarized below), each option granted to Mr. Gad, Dr. Møller and Mr. Kruse on October 21, 2016 and December 14, 2016 under the 2015 Plan will fully vest and become immediately exercisable.

In the event Mr. Gad, Dr. Møller or Mr. Kruse's employment is terminated by us without "cause" or by either Mr. Gad, Dr. Møller or Mr. Kruse for "good reason", or by their "retirement" or "disability", as such terms are defined in the 2015 Plan (with respect to good reason, as summarized below), or by death, the options granted to Mr. Gad, Dr. Møller and Mr. Kruse on December 14, 2016 will continue to vest and become exercisable in accordance with our Standard Vesting Schedule. However, if Mr. Gad, Dr. Møller or Mr. Kruse's employment is terminated for cause or by either Mr. Gad, Dr. Møller or Mr. Kruse voluntarily (other than for retirement), the options granted to Mr. Gad, Dr. Møller and Mr. Kruse on December 14, 2016 will terminate immediately and will not become exercisable. For Mr. Gad and Mr. Kruse's October 21, 2016 option award, upon termination of employment, the option will be exercisable for three months following termination, unless due to disability, in which case the option will remain exercisable for twelve months following termination, or death, in which case the option will remain exercisable for six months.

As defined in the 2015 Plan:

- "change of control" generally means (1) the acquisition by a person or entity of more than 50% of our combined voting power (except a change in ownership as a result of a private financing of us that is approved by the Board), (2) the change in effective control of us which occurs on the date that a majority of members of the Board is replaced during any twelve (12) month period by directors whose appointment or election is not endorsed by a majority of the members of the board prior to the date of the appointment or election (if any person or entity is considered to be in effective control of us, the acquisition of additional control of us by the same person or entity will not be considered a change in control), and (3) the acquisition by a person or entity of a substantial portion of our assets with a total gross fair market value equal to or more than 50% of the total gross fair market value of all of our assets immediately prior to such acquisition;
- "cause" generally means abuse of alcohol or another drug while performing his or her duties as an employee of us, or a breach of or failure or refusal by participant to comply with any material provision of his or her employment agreement or arrangement with us if not cured within ten (10) days after written notice thereof from us; and
- "good reason" generally means, during the term of the participant's employment relationship with us, without the participant's written consent, we cause a material reduction in base salary or compensation and bonus opportunity, a relocation of participant's principal place of employment by more than 50 miles, any material breach by us of any provision in the participant's employment agreement or arrangement or other agreements, our failure to obtain an agreement from any successor to us to assume and agree to perform a participant's employment agreement or arrangement in the same manner and to the same extent that we would be required to perform if no succession had taken place (except where such assumptions occurs by operation of law), a material, adverse change in the participant's title, authority, duties, or responsibilities (except temporary change while participant is physically or mentally incapacitated or as required by applicable law), or a material change in the reporting structure applicable to the participant.

All options expire 10 years from the date of grant. None of the options granted to the named executive officers provide for tax-reimbursements or tax gross-ups. To date, our board of directors has not granted any options to our named executive officers in 2017.

### Outstanding Equity Awards at 2017 Year End

The following table sets forth information regarding outstanding equity awards held by our named executive officers as of December 31, 2017:

Name	Number of Securities Underlying Unexercised Options Exercisable (#)	Number of Securities Underlying Unexercised Options Unexercisable (#)	Option Exercise Price (\$/share)	Option Expiration Date
Thomas Gad <sup>(1)</sup>	312,500	187,500	\$ 2.00	June 9, 2025
	48,417	117,583	\$ 4.38	October 20, 2026
	4,000	12,000	\$ 8.50	December 13, 2026
Dr. Claus Juan Møller San Pedro <sup>(2)</sup>	312,500	187,500	\$ 2.00	June 9, 2025
	58,333	141,667	\$ 4.38	October 20, 2026
	4,500	13,500	\$ 8.50	December 13, 2026
Bo Kruse <sup>(3)</sup>	187,500	112,500	\$ 2.00	June 9, 2025
	38,792	94,208	\$ 4.38	October 20, 2026
	3,500	10,500	\$ 8.50	December 13, 2026

- (1) These options were granted on June 10, 2015, October 21, 2016 and December 14, 2016, respectively, vested as to 25% of the shares on June 10, 2016, October 21, 2017 and December 14, 2017, respectively, and vest thereafter as to 2.0833% of the shares in equal monthly installments through June 10, 2020, October 21, 2021 and December 14, 2021.
- (2) These options were granted on June 10, 2015, October 21, 2016 and December 14, 2016, respectively, vested as to 25% of the shares on June 10, 2016, October 21, 2017 and December 14, 2017, respectively, and vest thereafter as to 2.0833% of the shares in equal monthly installments through June 10, 2020, October 21, 2021 and December 14, 2021. Dr. Møller transferred all shares underlying the June 10, 2016 option award upon receipt to CM Holdings 2015 ApS, of which Dr. Møller is the sole owner.
- (3) These options were granted on June 10, 2015, October 21, 2016 and December 14, 2016, respectively, vested as to 25% of the shares on June 10, 2016, October 21, 2017 and December 14, 2017, respectively, and vest thereafter as to 2.0833% of the shares in equal monthly installments through June 10, 2020, October 21, 2021 and December 14, 2021.

### Employment Agreements

#### Thomas Gad

In April 2016, we entered into a service agreement with Mr. Gad. The service agreement establishes Mr. Gad's title, his base salary, his eligibility for an annual bonus, and his eligibility for benefits and also provides for certain benefits upon termination of his employment under specified conditions. Mr. Gad is eligible to receive an annual bonus with a target of 50% of his base salary. Mr. Gad's employment under the service agreement continues until terminated by us or Mr. Gad. We may terminate Mr. Gad's employment for any reason with 12 months' notice and Mr. Gad may terminate his employment with 6 months' notice.

Under the terms of the service agreement, if Mr. Gad's employment is terminated by us without "cause", as defined in his service agreement, and subject to Mr. Gad's execution of a release in form and substance satisfactory to us, we have agreed to continue to pay his then-existing salary for a period of 12 months, and all benefits set forth in the service agreement, for one full year commencing with the day following the final day of the 12-month notice period such that the total amount in

severance pay shall be 24 months of his then-existing salary, starting from the date of such termination notice.

As defined in Mr. Gad's service agreement, "cause" means (1) Mr. Gad's fraudulent, unlawful, grossly negligent or willful misconduct in connection with his duties to us, (2) conduct by Mr. Gad which is materially injurious to the business or reputation of us or any of our affiliated entities or any of their respective partners or members, or (3) Mr. Gad's conviction of (or plea of *nolo contendere* to) a felony.

Mr. Gad has also agreed pursuant to his service agreement (1) not to compete with us in the United States, Denmark, or any other territory or country where we maintain employees, own property or otherwise conduct business, during his employment and for a period of (a) one year after the termination of his employment in the event that Mr. Gad terminates his employment or (b) six months after the termination of his employment in the event that we terminate Mr. Gad's employment, (2) not to solicit our employees during his employment and for a period of (a) one year after the termination of his employment in the event that Mr. Gad terminates his employment or (b) six months after the termination of his employment in the event that we terminate Mr. Gad's employment, (3) not to disclose our confidential and proprietary information and (4) to assign to us related intellectual property developed during the course of his employment.

#### **Dr. Claus Juan Møller San Pedro**

In March 2016, we entered into a service agreement with Dr. Møller. The service agreement establishes Dr. Møller's title, his base salary, his eligibility for an annual bonus, and his eligibility for benefits and also provides for certain benefits upon termination of his employment under specified conditions. Dr. Møller is eligible to receive an annual bonus with a target of 50% of his base salary. Dr. Møller's employment under the service agreement continues until terminated by us or Dr. Møller. We may terminate Dr. Møller's employment for any reason with 12 months' notice and Dr. Møller may terminate his employment with 6 months' notice.

Under the terms of the service agreement, if Dr. Møller's employment is terminated by us without "cause", as defined in his service agreement, and subject to Dr. Møller's execution of a release in form and substance satisfactory to us, we have agreed to continue to pay his then-existing salary for a period of 12 months, and all benefits set forth in the service agreement, for one full year commencing with the day following the final day of the 12-month notice period such that the total amount in severance pay shall be 24 months of his then-existing salary, starting from the date of such termination notice.

As defined in Dr. Møller's service agreement, "cause" means (1) Dr. Møller's fraudulent, unlawful, grossly negligent or willful misconduct in connection with his duties to us, (2) conduct by Dr. Møller which is materially injurious to the business or reputation of us or any of our affiliated entities or any of their respective partners or members, or (3) Dr. Møller's conviction of (or plea of *nolo contendere* to) a felony.

Dr. Møller has also agreed pursuant to the service agreement (1) not to compete with us in the United States, Denmark, or any other territory or country where we maintain employees, owns property or otherwise conducts business, during his employment and for a period of (a) one year after the termination of his employment in the event that Dr. Møller terminates his employment or (b) six months after the termination of his employment in the event that we terminate Dr. Møller's employment, (2) not to solicit our employees during his employment and for a period of (a) one year after the termination of his employment in the event that Dr. Møller terminates his employment or (b) six months after the termination of his employment in the event that we terminate Dr. Møller's employment, (3) not to disclose our confidential and proprietary information and (4) to assign to us related intellectual property developed during the course of his employment.



## **Bo Kruse**

In January 2016, we entered into a service agreement with Mr. Kruse. The service agreement establishes Mr. Kruse's title, his base salary, his eligibility for an annual bonus, and his eligibility for benefits and also provides for certain benefits upon termination of his employment under specified conditions. Mr. Kruse is eligible to receive an annual bonus with a target of 50% of his base salary. Mr. Kruse's employment under the service agreement continues until terminated by us or Mr. Kruse. We may terminate Mr. Kruse's employment for any reason with 12 months' notice and Mr. Kruse may terminate his employment with 6 months' notice.

Under the terms of the service agreement, if Mr. Kruse's employment is terminated by us without "cause", as defined in his service agreement, we have agreed to continue to pay his then-existing salary for a period of 12 months, and all benefits set forth in the service agreement, for one full year commencing with the day following the final day of the 12-month notice period such that the total amount in severance pay shall be 24 months of his then-existing salary, starting from the date of such termination notice.

As defined in Mr. Kruse's service agreement, "cause" means actions on the part of Mr. Kruse which constitute gross negligence or willful misconduct in performance or non-performance of his duties or material breach of the services agreement by Mr. Kruse as long as such material breach is not cause by us.

Mr. Kruse has also agreed pursuant to the service agreement (1) not to disclose our confidential and proprietary information and (2) to assign to us related intellectual property developed during the course of his employment.

## **Stock Option and Other Compensation Plans**

The equity incentive plans described in this section are the 2015 Plan and the ESPP. Following the closing of this offering, we expect to grant equity awards to eligible participants only under these plans or successor plans.

### **2015 Plan**

Our board of directors and stockholders have approved and adopted the 2015 Plan, which provides for the grant of incentive stock options, within the meaning of Section 422 of the Code, to our employees and any parent and subsidiary corporations' employees, and for the grant of nonstatutory stock options, restricted stock, restricted stock units and stock appreciation rights to our employees, directors and consultants and our parent and subsidiary corporations' employees and consultants.

**Stock Subject to the 2015 Plan.** Currently, a total of 4,500,000 shares of our common stock are reserved for issuance pursuant to the 2015 Plan, of which 2,281,000 are available for further grant.

**Automatic Share Reserve Increase.** Subject to the provisions of the 2015 Plan, the number of shares available for issuance under the 2015 Plan will be increased on the first day of each fiscal year so that the total number of shares available for issuance under the 2015 Plan shall be a number equal to six percent of the issued and outstanding Shares on the last day of the immediately preceding fiscal year.

**Lapsed Awards.** If an award expires or becomes unexercisable without having been exercised in full, is surrendered pursuant to an exchange program, or, with respect to restricted stock or restricted stock units, is forfeited or repurchased due to failure to vest, the unpurchased shares, or for awards other than stock options or stock appreciation rights, the forfeited or repurchased shares will become available for future grant or sale under the 2015 Plan. With respect to stock appreciation rights, the net shares issued will cease to be available under the 2015 Plan and all remaining shares will

remain available for future grant or sale under the 2015 Plan. Shares used to pay the exercise price of an award or satisfy the tax withholding obligations related to an award will become available for future grant or sale under the 2015 Plan. To the extent an award is paid out in cash rather than shares, such cash payment will not result in reducing the number of shares available for issuance under the 2015 Plan.

**Plan Administration.** Our board of directors or one or more committees appointed by our board of directors, or the Plan Administrator, will administer the 2015 Plan. In the case of awards intended to qualify as "performance-based compensation" within the meaning of Section 162(m) of the Code, the committee will consist of two or more "outside directors" within the meaning of Section 162(m). Subject to the provisions of the 2015 Plan, the Plan Administrator has the power to administer the 2015 Plan, including but not limited to, the power to interpret the terms of the 2015 Plan and awards granted under it, to create, amend and revoke rules relating to the 2015 Plan, including rules and regulations relating to sub-plans, and to determine the terms of the awards, including the exercise price, the number of shares subject to each such award, the exercisability of the awards and the form of consideration, if any, payable upon exercise. The Plan Administrator also has the authority to institute an exchange program under which the exercise price of an existing award is reduced or increased, participants may transfer outstanding awards to a financial institution or other person or entity selected by the Plan Administrator and outstanding awards may be surrendered in exchange for awards of the same type which may have a higher or lower exercise price or different terms, awards of a different type and/or cash.

**Stock Options.** Incentive stock options and nonstatutory stock options may be granted under the 2015 Plan. The exercise price of options granted under the 2015 Plan must at least be equal to the fair market value of our common stock on the date of grant. The term of an incentive stock option may not exceed 10 years, except that with respect to any participant who owns more than 10% of the voting power of all classes of our outstanding stock, the term must not exceed five years and the exercise price must equal at least 110% of the fair market value on the grant date. The Plan Administrator will determine the methods of payment of the exercise price of an option, which may include cash, shares or other property acceptable to the Plan Administrator, as well as other types of consideration permitted by applicable law. After the termination of service of an employee, director or consultant, he or she may exercise his or her option for the period of time stated in his or her option agreement. Generally, if termination is due to disability, the option will remain exercisable for 12 months, and if the termination is due to death, the option will remain exercisable for six months. In all other cases, the option will generally remain exercisable for the earlier of (i) three months following the termination of service, or (ii) the expiration of the term of the option. However, in no event may an option be exercised later than the expiration of its term. Subject to the provisions of the 2015 Plan, the Plan Administrator determines the other terms of options.

**Stock Appreciation Rights.** Stock appreciation rights may be granted under the 2015 Plan. Stock appreciation rights allow the recipient to receive the appreciation in the fair market value of our common stock between the exercise date and the date of grant. Stock appreciation rights may not have a term exceeding 10 years. During employment or after the termination of service of an employee, director or consultant, he or she may exercise his or her stock appreciation right for the period of time stated in his or her option agreement. However, in no event may a stock appreciation right be exercised later than the expiration of its term. Subject to the provisions of the 2015 Plan, the Plan Administrator determines the other terms of stock appreciation rights, including when such rights become exercisable and whether to pay any increased appreciation in cash or with shares of our common stock, or a combination thereof, except that the per share exercise price for the shares to be issued pursuant to the exercise of a stock appreciation right will be no less than 100% of the fair market value per share on the date of grant.

**Restricted Stock.** Restricted stock awards may be granted under the 2015 Plan. Restricted stock awards are grants of shares of our common stock that vest in accordance with terms and conditions established by the Administrator. The Plan Administrator will determine the number of shares of restricted stock granted to any employee, director or consultant and, subject to the provisions of the 2015 Plan, will determine the terms and conditions of such awards. The Plan Administrator may impose whatever conditions to vesting it determines to be appropriate. For example, the Plan Administrator may set restrictions based on the achievement of specific performance goals or continued service to us; provided, however, that the Plan Administrator, in its sole discretion, may accelerate the time at which any restrictions will lapse or be removed. Recipients of restricted stock awards generally will have voting and dividend rights with respect to such shares upon grant without regard to vesting, unless the Plan Administrator provides otherwise.

**Restricted Stock Units.** Restricted stock units may be granted under the 2015 Plan. Restricted stock units are bookkeeping entries representing an amount equal to the fair market value of one share of our common stock. Subject to the provisions of the 2015 Plan, the Plan Administrator will determine the terms and conditions of restricted stock units, including the vesting criteria (which may include accomplishing specified company-wide, business unit or individual performance criteria or continued service to us) and the form and timing of payment. Notwithstanding the foregoing, the Plan Administrator, in its sole discretion, may accelerate the time at which any restricted stock units will vest. Restricted stock units may be settled in cash, shares or a combination of both.

**Outside Directors.** The 2015 Plan provides that all non-employee directors are eligible to receive all types of awards other than incentive stock options under the 2015 Plan.

**Non-transferability of Awards.** Except as provided in the 2015 Plan or as the Plan Administrator determines, awards may not be sold, transferred, pledged, assigned, or otherwise alienated or hypothecated until the end of the applicable period of restriction, and such shares evidenced by a stock certificate will contain a legend referencing the shares' substantial risk of forfeiture restrictions.

**Certain Adjustments.** In the event of certain changes in our capitalization or change in our corporate structure, as described in the 2015 Plan, to prevent diminution or enlargement of the benefits or potential benefits available under the 2015 Plan, the Plan Administrator will adjust the number and class of shares that may be delivered under the 2015 Plan and/or the number, class and price of shares covered by each outstanding award. In the event of our proposed liquidation or dissolution, the Plan Administrator will notify participants as soon as practicable prior to the proposed transaction and all awards will terminate immediately prior to the consummation of such proposed transaction.

**Merger or Change in Control.** The 2015 Plan provides that in the event of a merger or change in control, as defined under the 2015 Plan, each outstanding award will be treated as the Plan Administrator determines, except that if a successor corporation or its parent or subsidiary does not assume or substitute an award for any outstanding award (or a portion thereof), then such award will fully vest, all restrictions on the shares subject to such award will lapse, all performance goals or other performance-based vesting criteria applicable to the shares subject to such award will be deemed achieved at 100% of target levels. If an award is not assumed by the successor in accordance with the terms of the award, all of the shares subject to such award will become fully exercisable, if applicable, for a specified period prior to the transaction. The award will then terminate upon the expiration of the specified period of time.

**Tax Compliance.** Awards under the 2015 Plan generally will be designed and operated in such a manner that they are either exempt from the application of, or comply with, the requirements of Code Section 409A, The 2015 Plan and each award agreement thereunder is intended to meet the requirements of Code Section 409A and generally will be construed and interpreted in accordance with

such intent. To the extent that an award or payment, or the settlement or deferral thereof, is subject to Code Section 409A the award will be granted, paid, settled or deferred in a manner that will meet the requirements of Code Section 409A, such that the grant, payment, settlement or deferral will not be subject to the additional tax and interest applicable under Code Section 409A, except as otherwise determined in the sole discretion of the Administrator.

In addition, prior to the delivery of any shares or cash pursuant to an award (or exercise thereof), we have the power and the right to deduct or withhold, or require a participant to remit to us an amount sufficient to satisfy federal, state, local, foreign income, payroll or other taxes (including the participant's FICA obligation) required to be withheld with respect to such award (or exercise thereof).

The Administrator, in its sole discretion may permit a participant to satisfy such tax withholding obligation, in whole or in part by (without limitation) (i) paying cash, (ii) electing to have us withhold otherwise deliverable shares having a fair market value equal to the minimum statutory amount required to be withheld, (iii) delivering already-owned shares having a fair market value equal to the statutory amount required to be withheld, or (iv) selling a sufficient number of shares otherwise deliverable to the participant equal to the amount required to be withheld. The amount of the withholding requirement will be deemed to include any amount which the Administrator agrees may be withheld at the time the election is made, not to exceed the amount determined by using the maximum federal, state or local marginal income tax rates applicable to the participant with respect to the award on the date that the amount of tax to be withheld is to be determined. The fair market value of the shares to be withheld or delivered will be determined as of the date that the taxes are required to be withheld.

**Amendment, Termination.** The board of directors has the authority to amend, suspend or terminate the 2015 Plan provided such action will not impair the existing rights of any participant without such participant's consent. The 2015 Plan will automatically terminate in 2025, unless we terminate it sooner.

### **ESPP**

Our board of directors is currently considering the implementation of an employee stock purchase plan, or ESPP, to become effective upon completion of this offering. If adopted by our board of directors and approved by our stockholders, among other things, the ESPP will provide eligible employees with the ability to purchase shares of our common stock at a slight discount to the applicable closing sale price of our common stock. The ESPP will be described in further detail in this Registration Statement if it is adopted by our board of directors.

### **Other Benefit Plans**

We currently provide broad-based health and welfare benefits that are available to all of our U.S. employees, including our named executive officers, including health, life and disability insurance. We may adopt a 401(k) retirement plan for the benefit of our U.S. employees, including our named executive officers, in 2018.

We have established a retirement program for the employees of our Danish subsidiary pursuant to which all such employees can contribute an amount at their election from their base compensation and may receive contributions from our Danish subsidiary. Contributions from our Danish subsidiary were immaterial during the year ended December 31, 2016. In addition, health insurance benefits for our Danish employees are fully paid for by such employees. Our Danish subsidiary does not incur any costs for these health insurance benefits.

## **Limitations on Liability and Indemnification**

As permitted by the General Corporation Law of the State of Delaware, or DGCL, we expect our board of directors and stockholders to adopt provisions in our certificate of incorporation, which will become effective as of the closing date of this offering, that limit or eliminate the personal liability of our directors. Our certificate of incorporation, which will become effective as of the closing date of this offering, limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the DGCL and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty. However, these provisions do not eliminate or limit the liability of any of our directors:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;
- for voting for or assenting to unlawful payments of dividends, stock repurchases or other distributions; or
- for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the DGCL is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the DGCL.

In addition, our certificate of incorporation, which will become effective as of the closing date of this offering, provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers specified liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers. In addition, we have entered into indemnification agreements with each of our executive officers and directors. With respect to Dr. Buschle, Dr. Healy and Dr. Tyagi, we have also agreed to indemnify their respective affiliated funds which have designated them to be members of our board of directors. These indemnification agreements require us, among other things, to indemnify each such director (and their affiliated funds) or executive officer for certain expenses, including attorneys' fees, judgments, fines and settlement amounts, incurred by him or her in any action or proceeding arising out of his or her service as one of our directors or officers.

Some of our non-employee directors may, through their relationships with their employers, be insured or indemnified against specified liabilities incurred in their capacities as members of our board of directors.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, executive officers or persons controlling us, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

## **Rule 10b5-1 Sales Plans**

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. The

director or officer may amend or terminate the plan in some circumstances. Our directors and executive officers may also buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

## Director Compensation

The following table sets forth information regarding compensation paid to our non-employee directors during the fiscal year ended December 31, 2017.

<u>Name</u>	<u>Year</u>	<u>Fees Earned or Paid in Cash (\$)</u>	<u>Option Awards (\$)</u>	<u>Total (\$)(1)</u>
Johan Wedell-Wedellsborg(1)	2017	\$ 0	\$ 0	\$ 0
Dr. Gregory Raskin(1)	2017	\$ 0	\$ 0	\$ 0

- (1) As of December 31, 2017, the aggregate number of shares of our common stock subject to each non-employee director's outstanding option awards was 36,000 shares for each of Mr. Wedell-Wedellsborg and Dr. Raskin, and no other non-employee directors held any stock options or other equity-based awards. Each of Mr. Wedell-Wedellsborg and Dr. Raskin were granted an option to purchase 36,000 shares of our common stock on October 21, 2016. These options vest over three years, with one-third of the shares of common stock underlying each option vesting at grant, and the remaining two-thirds vesting one thirty-sixth each month over the three years ending on October 21, 2019, subject to Mr. Wedell-Wedellsborg's and Dr. Raskin's continued service through such dates and unless vesting is accelerated pursuant to the terms of the grant. As of December 31, 2017, there were no other option awards outstanding and held by our non-employee directors.

Prior to the completion of this offering, we did not have a formal non-employee director compensation policy. In 2016, we granted options to purchase 36,000 shares of common stock to each of Mr. Wedell-Wedellsborg and Dr. Raskin, respectively, with an exercise price of \$4.38 per share. These options vest over three years, with one-third of the shares of common stock underlying each option vesting at grant and the remaining two-third vesting one thirty-sixth each month over the three years ending on October 21, 2019, subject to Mr. Wedell-Wedellsborg's and Dr. Raskin's continued service through such dates, and unless vesting is accelerated pursuant to the terms of the grant. As of December 31, 2017, there were no other stock awards or option awards outstanding and held by our non-employee directors.

We reimburse our non-employee directors for reasonable travel and out-of-pocket expenses incurred in connection with attending board of directors and committee meetings. The compensation that we pay to our executive management and employee directors is discussed earlier in this "Executive and Director Compensation" section. Except for fees and stock options, we do not provide our independent directors with any other form of compensation.

### ***Non-Employee Director Compensation Policy***

In connection with this offering, our board of directors is considering adopting a policy with respect to the compensation payable to our non-employee directors, which will become effective upon the completion of this offering. If adopted by our board of directors and approved by our stockholders, under this policy, each non-employee director will be eligible to receive compensation for his or her service on the board of directors and for service on each committee on which the director is a member,

which will consist of annual cash retainers and equity awards. Our non-employee directors will receive the following annual cash retainers for their service in 2018:

<u>Position</u>	<u>Retainer</u>
Board Member	\$
Board Chairperson	\$
Audit Committee Chair	\$
Compensation Committee Chair	\$
Nominating and Corporate Governance Committee Chair	\$
Audit Committee Member	\$
Compensation Committee Member	\$
Nominating and Corporate Governance Committee Member	\$

Stock option grants for non-employee directors will consist of (i) an initial stock option award with respect to                      shares granted at the first regularly scheduled board meeting held on or after a director's first appointment or election to our board of directors and vesting in equal monthly installments until the third anniversary of the date of grant, and (ii) an annual stock option award with respect to                      shares granted on the date of the first board meeting held following our annual stockholders meeting in each year commencing in 2018 and vesting on the first anniversary of the date of grant. The vesting of the initial and annual stock option grants are subject to the non-employee director's continued service on our board of directors.

Directors may be reimbursed for travel, food, lodging and other expenses directly related to their service as directors. Directors are also entitled to the protection provided by their indemnification agreements and the indemnification provisions in our current certificate of incorporation and bylaws, as well as our amended and restated certificate of incorporation and amended and restated bylaws that will become effective upon the completion of this offering.

**CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS**

The following is a description of transactions since our inception on April 30, 2015, to which we have been a party, in which the amount involved exceeded \$120,000, and in which any of our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest. We refer to such transactions as "related party transactions" and such persons as "related parties." With the approval of our board of directors, we have engaged in the related party transactions described below. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, from unaffiliated third parties.

Other than as described below and other than compensation arrangements, which are described where required under the section herein entitled "Executive and Director Compensation", there have not been, nor are there any currently proposed, transactions or series of similar transactions to which we have been or will be a party.

**Sales of Securities**

In August 2015, we issued and sold 5,010,000 shares of our common stock at a price per share of \$0.20, for an aggregate purchase price of approximately \$1,002,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the approximate aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
WG Biotech ApS	5,010,000	\$ 1,002,000
<b>Total</b>	<b>5,010,000</b>	<b>\$ 1,002,000</b>

In November 2015, we issued and sold 1,027,397 shares of our common stock at a purchase price of \$4.38 per share for an aggregated purchase price of approximately \$4,500,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the approximate aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
Weco Group A/S	114,155	\$ 500,000
Peter Bang Holdings ApS	913,242	\$ 4,000,000
<b>Total</b>	<b>1,027,397</b>	<b>\$ 4,500,000</b>

In December 2015, we issued and sold 1,027,487 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$4,500,400. The following



table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
Weco Group A/S	114,155	\$ 500,000
Weco Group A/S	136,986	\$ 600,000
<b>Affiliates of Executive Officers and Directors</b>		
CM Holding 2015 IVS	68,493	\$ 300,000
<b>Total</b>	<b>319,634</b>	<b>\$ 1,400,000</b>

In March 2016, we issued and sold 570,776 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$2,500,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
Weco Group A/S	228,310	\$ 1,000,000
<b>Affiliates of Executive Officers and Directors</b>		
CM Holding 2015 ApS	114,155	\$ 500,000
<b>Total</b>	<b>342,465</b>	<b>\$ 1,500,000</b>

In April 2016, we issued and sold 1,261,412 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$5,525,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
Peter Bang Holdings ApS	342,465	\$ 1,500,000
Weco Group A/S	114,155	\$ 500,000
Weco Group A/S	114,155	\$ 500,000
<b>Total</b>	<b>570,775</b>	<b>\$ 2,500,000</b>

In May 2016, we issued and sold 515,204 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$2,256,600. The following table sets

forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b><i>Affiliates of Executive Officers and Directors</i></b>		
CM Holding 2015 ApS	45,662	\$ 200,000
<b>Total</b>	<b>45,662</b>	<b>\$ 200,000</b>

In June 2016, we issued and sold 890,406 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$3,900,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b><i>5% Stockholders</i></b>		
Weco Group A/S	136,986	\$ 600,000
<b><i>Executive Officers and Directors</i></b>		
Bo Kruse	57,077	\$ 250,000
<b><i>Affiliates of Executive Officers and Directors</i></b>		
Toluha ApS	45,662	\$ 200,000
<b>Total</b>	<b>239,725</b>	<b>\$ 1,050,000</b>

In July 2016, we issued and sold 187,214 shares of our common stock at a price per share of \$4.38, for an aggregate purchase price of approximately \$820,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b><i>5% Stockholders</i></b>		
Peter Bang Holdings ApS	187,214	\$ 820,000
<b>Total</b>	<b>187,214</b>	<b>\$ 820,000</b>

In December 2016, we issued and sold 578,982 shares of our common stock at a purchase price of \$8.50 per share for an aggregate purchase price of approximately \$4,921,400. The following table sets

forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
Weco Group A/S	294,115	\$ 2,500,000
<b>Affiliates of Executive Officers and Directors</b>		
CM Holding 2015 ApS	70,588	\$ 600,000
<b>Total</b>	<b>364,703</b>	<b>\$ 3,100,000</b>

In January 2017, we issued and sold 1,016,486 shares of our common stock at a purchase price of \$8.50 per share for an aggregate purchase price of approximately \$8,640,200. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
Peter Bang Holding ApS	235,294	\$ 2,000,000
Weco Group A/S	29,411	\$ 250,000
<b>Total</b>	<b>264,705</b>	<b>\$ 2,250,000</b>

In February 2017, we issued and sold 117,353 shares of our common stock at a purchase price of \$8.50 per share for an aggregate purchase price of approximately \$997,500. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>Executive Officers and Directors</b>		
Dr. Gregory Raskin	5,882	\$ 50,000
<b>Total</b>	<b>5,882</b>	<b>\$ 50,000</b>

In October 2017, we issued and sold 5,347,568 shares of our common stock at a purchase price of \$9.35 per share for an aggregate purchase price of approximately \$50,000,000. The following table

sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
HBM Healthcare Investments (Cayman) Ltd.	2,139,037	\$ 20,000,000
Memorial Sloan Kettering Cancer Center	320,855	\$ 3,000,000
Peter Bang Holding ApS	404,582	\$ 3,782,842
Weco Group A/S	234,759	\$ 2,195,000
<b>Affiliates of Executive Officers and Directors</b>		
CM Holding 2015 ApS	42,780	\$ 400,000
<b>Total</b>	<b>3,142,013</b>	<b>\$ 29,377,842</b>

In November 2017, we issued and sold 3,208,552 shares of our common stock at a purchase price of \$9.35 per share for an aggregate purchase price of \$30,000,000. The following table sets forth the number of shares of our common stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

<u>Name</u>	<u>Shares of Common Stock Purchased</u>	<u>Aggregate Purchase Price</u>
<b>5% Stockholders</b>		
HBM Healthcare Investments (Cayman) Ltd.	347,058	\$ 3,245,000
Sofinnova Venture Partners X, L.P.	1,604,278	\$ 15,000,000
Weco Group A/S	166,310	\$ 1,555,000
<b>Total</b>	<b>2,117,646</b>	<b>\$ 19,800,000</b>

### Registration Rights Agreements

We are a party to certain registration rights agreements dated as of October 13, 2017 and November 17, 2017, collectively referred to herein as the Registration Rights Agreements, with certain holders of our common stock, including some of our directors and 5% stockholders and their affiliates and entities affiliated with our directors. The Registration Rights Agreements provide these holders the right, following the completion of this offering, to demand that we file a registration statement or request that their shares be covered by a registration statement that we are otherwise filing. See the section herein entitled "Description of Capital Stock—Registration Rights" for additional information regarding these registration rights.

### Director and Executive Officer Compensation

See the section herein entitled "Executive and Director Compensation" for a discussion of payments and options granted to our named executive officers and non-employee directors.

### Employment Agreements

We have entered into employment agreements with our executive officers. For more information regarding these agreements, see the section herein entitled "Executive and Director Compensation—Narrative Disclosure to Summary Compensation Table."

## **Indemnification Agreements with Officers and Directors and Directors' and Officers' Liability Insurance**

In connection with this offering, we have entered into indemnification agreements with each of our executive officers and directors. In addition, we intend to enter into such indemnification agreements with any new executive officers and directors. The indemnification agreements, our amended and restated certificate of incorporation and our amended and restated bylaws that will become effective upon the completion of this offering will require us to indemnify our directors to the fullest extent not prohibited by DGCL. Subject to certain limitations, our amended and restated bylaws also require us to advance expenses incurred by our directors and officers.

### **MSK License**

On August 20, 2015, we entered into the MSK License with MSK, which grants us a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments. See the section herein entitled "Business—Intellectual Property—MSK Agreements" for a description of this agreement.

### **MSK CD33 License Agreement**

On November 13, 2017, we entered into the MSK CD33 License with MSK, which grants us a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments in connection with certain CD33 antibodies generated in a specific principal investigator's laboratory at MSK and constructs thereof. The MSK CD33 License is exclusive with respect to MSK's rights in such patent rights and tangible materials within such know-how, and nonexclusive with respect to such know-how and related intellectual property rights. See the section herein entitled "Business—Intellectual Property—MSK Agreements" for a description of this agreement.

### **Stockholders Agreement**

We are party to a fourth amended and restated stockholders' agreement with the holders of our common stock, including our 5% stockholders and their affiliates and entities affiliated with some of our directors, providing for, among other things, specified voting with respect to the election of directors. This agreement will terminate upon the closing of this offering.

### **Share Subscription, Funding Commitment and Call Option Agreement**

In August 2015, we entered into a Share Subscription, Funding Commitment and Call Option Agreement with WG Biotech ApS for the sale of 5,010,000 shares of our common stock at a price per share of \$0.20, for an aggregate purchase price of approximately \$1,002,000.

### **Securities Purchase Agreements**

We are a party to certain securities purchase agreements dated as of October 13, 2017 and November 17, 2017, with certain holders of our common stock, including some of our directors and 5% stockholders and their affiliates and entities affiliated with our directors, pursuant to which we issued and sold shares of our common stock at a purchase price of \$9.35 per share for an aggregate purchase price of approximately \$80,000,000. See the section herein entitled "Certain Relationships and Related Party Transactions—Sales of Securities" for additional information regarding the sale of these securities.

**Policies and Procedures for Related Party Transactions**

In connection with this offering, we plan to adopt a written policy, effective upon completion of this offering, that requires all future transactions between us and any director, executive officer, holder of 5% or more of any class of our capital stock or any member of the immediate family of, or entities affiliated with, any of them, or any other related persons, as defined in Item 404 of Regulation S-K, or their affiliates, in which the amount involved is equal to or greater than \$120,000, be approved in advance by our audit committee. Any request for such a transaction must first be presented to our audit committee for review, consideration and approval. In approving or rejecting any such proposal, our audit committee is to consider the relevant facts and circumstances available and deemed relevant to the audit committee, including, but not limited to, the extent of the related party's interest in the transaction, and whether the transaction is on terms no less favorable to us than terms we could have generally obtained from an unaffiliated third party under the same or similar circumstances.

## PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock as of December 31, 2017 by:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

The column entitled "Percentage of Shares Beneficially Owned—Before Offering" is based on a total of 28,093,666 shares of our common stock outstanding as of December 31, 2017. The column entitled "Percentage of Shares Beneficially Owned—After Offering" is based on \_\_\_\_\_ shares of our common stock to be outstanding after this offering, including the \_\_\_\_\_ shares of our common stock that we are selling in this offering, but not including any additional shares issuable upon exercise of outstanding options or any exercise by the underwriters of their option to purchase additional shares.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options that are currently exercisable or exercisable within 60 days after December 31, 2017, are considered outstanding and beneficially owned by the person holding the options for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investment power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set forth below, the address of each beneficial owner is c/o Y-mAbs Therapeutics, Inc., 750 Third Avenue, 9th Floor, New York, NY 10017.

<u>Name and Address of Beneficial Owner</u>	<u>Number of Shares Beneficially Owned</u>	<u>Percentage of Shares Beneficially Owned</u>	
		<u>Before Offering</u>	<u>After Offering</u>
<b>5% Stockholders</b>			
WG Biotech ApS Rungsted Strandvej 113 DK 0960 Rungsted Kyst, Denmark Attn: Johan Wedell-Wedellsborg	5,010,000	17.83%	
Memorial Sloan Kettering Cancer Center Office of Technology Development 1275 York Avenue Box 524 New York, NY, 10065 Attn: Vice President	2,749,284	9.79%	
HBM Healthcare Investments (Cayman) Ltd.(1) Governors Square Suite #4-212-2 23 Lime Tree Bay Avenue West Bay, Grand Cayman Cayman Islands Attention: Jean-Marc LeSieur	2,486,095	8.85%	
Dr. Nai-Kong V. Cheung 425 East 58 Street Apt. 34D New York, NY 10022	2,426,400	8.64%	
Peter Bang Holding ApS Richelieus Alle 8 DK 2900 Hellerup, Denmark Attn: Peter Bang	2,282,797	8.13%	
Weco Group A/S Rungsted Strandvej 113 DK 2960 Rungsted Kyst, Denmark Attn: Johan Wedell-Wedellsborg	1,683,496	5.99%	
Sofinnova Venture Partners X, L.P. 3000 Sand Hill Road Building 4-Suite 250 Menlo Park, CA 94025 Attention: Hooman Shahlavi, Partner & General Counsel	1,604,278	5.71%	
<b>Named Executive Officers and Directors</b>			
Thomas Gad(2)	6,593,333	23.47%	
Dr. Claus Juan Møller San Pedro(3)	1,246,928	4.44%	
Bo Kruse(4)	605,494	2.16%	
Johan Wedell-Wedellsborg(5)	6,716,162	23.91%	
Dr. Gregory Raskin(6)	28,548	*	
Dr. Michael Buschle(7)	—	*	
Dr. James I. Healy(8)	1,604,278	5.71%	
Dr. Ashutosh Tyagi(9)	962,566	3.43%	
David N. Gill	—	*	
<i>All Current Executive Officers and Directors as a Group (9 persons)(10)</i>	17,757,309	63.92%	

\* Represents beneficial ownership of less than 1% of our outstanding shares of common stock.

- (1) The board of directors of HBM Healthcare Investments (Cayman) Ltd. has sole voting and investment power with respect to the shares by held by such entity. The board of directors of HBM Healthcare Investments (Cayman) Ltd. is comprised of Jean-Marc LeSieur, Richard Coles, Sophia Harris, Dr. Andreas Wicki, Paul Woodhouse and Mark Kronenfeld, none of whom has individual voting or



investment power with respect to such shares, and each disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. The address for HBM Healthcare Investments (Cayman) Ltd. is Governor's Square, Suite #4-212-2, 23 Lime Tree Bay Avenue, West Bay, Grand Cayman, Cayman Islands. The percentage of shares beneficially owned after this offering would be 13.1%, assuming the purchase of all of the shares that HBM Healthcare Investments (Cayman) Ltd. has indicated an interest in purchasing in this offering.

- (2) Includes (i) 1,190,000 shares of common stock owned by GAD Enterprises LLC, of which Mr. Gad is the sole member and manager and as such Mr. Gad has sole voting and dispositive power with respect to such shares, and (ii) 393,333 shares of common stock underlying options that are exercisable as of December 31, 2017 or will become exercisable within 60 days after such date. Also includes 5,010,000 shares of common stock held by WG Biotech ApS, in which Mr. Gad owns a 21.96% equity interest. Mr. Gad expressly disclaims beneficial ownership of the shares held by WG Biotech ApS, except to the extent of his pecuniary interest therein.
- (3) Includes (i) 841,678 shares of common stock owned by CM Holding 2015 ApS, Dr. Møller's personal holding company of which Dr. Møller is the sole owner and as such Dr. Møller has sole voting and dispositive power with respect to such shares, and (ii) 405,250 shares of common stock underlying options that are exercisable as of December 31, 2017 or will become exercisable within 60 days after such date.
- (4) Includes (i) 300,000 shares of common stock owned directly by Mr. Kruse, (ii) 57,077 shares of common stock owned by Investeringselskabet G.H. ApS, Mr. Kruse's personal holding company of which Mr. Kruse is the sole owner and as such has the sole voting and dispositive power with respect to such shares, and (iii) 248,417 shares of common stock underlying options that are exercisable as of December 31, 2017 or will become exercisable within 60 days after such date.
- (5) Includes (i) 5,010,000 shares of common stock owned by WG Biotech ApS in which Mr. Wedell-Wedellsborg is the majority owner and as such has sole voting and dispositive power with respect to such shares, (ii) 1,683,496 shares owned by Weco Grup A/S of which Mr. Wedells-Wedellsborg is the owner and as such has sole voting and dispositive power with respect to such shares, and (iii) 22,666 shares of common stock underlying options that are exercisable as of December 31, 2017 or will become exercisable within 60 days after such date.
- (6) Includes 5,882 shares of common stock owned directly by Dr. Raskin. Also includes 22,666 shares of common stock underlying options that are exercisable as of December 31, 2017 or will become exercisable within 60 days after such date. Does not include 2,749,284 shares of common stock owned by MSK. Dr. Raskin is an employee of MSK and is MSK's representative on our Board of Directors. Dr. Raskin has no voting or dispositive power with respect the shares owned by MSK, and therefore, Dr. Raskin disclaims beneficial ownership of such shares except to the extent of his pecuniary interest arising as a result of his employment with MSK.
- (7) Dr. Buschle, a member of our board of directors, is a consultant with HBM Partners AG. HBM Partners AG provides investment advisory services to HBM Healthcare Investments (Cayman) Ltd. Dr. Buschle has no voting or investment power over the shares held by HBM Healthcare Investments (Cayman) Ltd., and disclaims beneficial ownership of such shares.
- (8) Includes 1,604,278 shares of common stock owned by Sofinnova Venture Partners X, L.P., or Sofinnova. Dr. Healy is a managing member of Sofinnova Management X, L.L.C., the General Partner of Sofinnova, and as such shares voting and dispositive power over such shares.
- (9) Includes 659,358 shares of common stock owned by Scopia Health Care International Master Fund LLP, or Scopia LLP, and 303,208 shares of common stock owned by Scopia Health Care LLC, or Scopia LLC. Dr. Tyagi is an employee of Scopia Capital Management, Inc., or Scopia Capital, an affiliate of both Scopia LLP and Scopia LLC. Dr. Tyagi disclaims beneficial ownership of such shares except to the extent of his pecuniary interest arising as a result of his employment with Scopia Capital.
- (10) Includes 1,092,332 shares of common stock underlying options that are exercisable as of December 31, 2017 or will become exercisable within 60 days after such date.

## DESCRIPTION OF CAPITAL STOCK

The following description of the rights of our common stock and convertible preferred stock is intended as a summary only and is qualified in its entirety by reference to our amended and restated certificate of incorporation and amended and restated bylaws, which will be filed as exhibits to the registration statement of which this prospectus is a part, and to the applicable provisions of the DGCL. The description of our capital stock reflects changes to our capital structure that will occur upon the completion of this offering.

Upon the closing of this offering and the filing of our certificate of incorporation to be effective upon completion of this offering, our authorized capital stock will consist of 100,000,000 shares of common stock, par value \$0.0001 per share, and 5,500,000 shares of preferred stock, par value \$0.0001 per share.

### **Common Stock**

#### ***Outstanding Shares***

Based on \_\_\_\_\_ shares of common stock outstanding as of \_\_\_\_\_, 2018 (including \_\_\_\_\_ shares of common stock subject to vesting), and upon the completion of this offering and the issuance of \_\_\_\_\_ shares of common stock in this offering, there will be \_\_\_\_\_ shares of common stock outstanding upon the closing of this offering. As of \_\_\_\_\_, 2018, we had approximately \_\_\_\_\_ record holders of our common stock. As of \_\_\_\_\_, 2018, there were \_\_\_\_\_ shares of common stock subject to outstanding options.

#### ***Voting Rights***

Each holder of common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Our certificate of incorporation and bylaws to be in effect upon the completion of this offering do not provide for cumulative voting rights. Because of this, the holders of a plurality of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose. With respect to matters other than the election of directors, at any meeting of the stockholders at which a quorum is present or represented, the affirmative vote of a majority of the voting power of the shares present in person or represented by proxy at such meeting and entitled to vote on the subject matter shall be the act of the stockholders, except as otherwise required by law. The holders of a majority of the stock issued and outstanding and entitled to vote, present in person or represented by proxy, shall constitute a quorum for the transaction of business at all meetings of the stockholders.

#### ***Dividends***

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds. For more information see the section of this prospectus captioned "Dividend Policy."

#### ***Liquidation***

In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

### ***Rights and Preferences***

Holders of common stock have no preemptive, conversion, subscription or other rights, and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate in the future.

### ***Fully Paid and Nonassessable***

All of our outstanding shares of common stock are, and the shares of common stock to be issued pursuant to this offering, when paid for, will be fully paid and nonassessable.

### ***Registration Rights***

The Registration Rights Agreements provide certain holders of our common stock, including some of our directors and 5% stockholders and their respective affiliates and entities affiliated with our directors, the right, following the completion of this offering, to require us to register these shares under the Securities Act under specified circumstances as described below. The shares subject to registration rights under the Registration Rights Agreements, or the registrable shares, will represent approximately % of our outstanding common stock after this offering, or % if the underwriters exercise their option to purchase additional shares. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act.

Under the Registration Rights Agreements, holders of registrable shares can demand that we file a registration statement or request that their shares be included on a registration statement that we are otherwise filing, in either case, registering the resale of their shares of common stock. These registration rights are subject to conditions and limitations, including the right, in certain circumstances, of the underwriters of an offering to limit the number of shares included in such registration and our right, in certain circumstances, not to effect a requested S-1 or S-3 registration during the period that is 60 days before our estimated date of filing of, and ending on a date that is 90 days (or 180 days in the case of our initial public offering) after the effective date of, a company-initiated registration statement.

The registration rights of any holder will terminate upon the earliest to occur of: (i) the date on which such holder holds no registrable shares, (ii) such time as Rule 144 or another similar exemption under the Securities Act is available for the sale of all of such holder's registrable shares without the requirement for us to be in compliance with the current publication information required under Rule 144(c)(1), and (iii) the fifth anniversary of this offering.

### ***Demand Registration Rights***

Beginning 180 days after the effective date of the registration statement of which this prospectus is a part, subject to specified limitations set forth in the Registration Rights Agreements, at any time the holder or holders of not less than a majority of our registrable securities, as defined in the Registration Rights Agreements, acting together, may demand in writing that we register the outstanding registrable securities under the Securities Act so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public of least \$10 million. We are not obligated to file a registration statement pursuant to this demand provision on more than two occasions, subject to specified exceptions.

In addition, at any time after we become eligible to file a registration statement on Form S-3 under the Securities Act, subject to specified limitations, a holder or holders of a majority of the registrable securities may demand in writing that we register on Form S-3 all or part of the registrable

securities held by them so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public of least \$10 million.

### ***Incidental Registration Rights***

If, at any time after the closing of this offering, we propose to file a registration statement to register any of our common stock under the Securities Act, either for our own account or for the account of any of our stockholders that are not holders of registrable securities, and on a form that would also permit the registration of registrable securities, the holders of our registrable securities are entitled to notice of registration and, subject to specified exceptions, we will be required to use our best efforts to register the registrable securities then held by them that they request that we register.

### ***Expenses of Registration***

Pursuant to the Registration Rights Agreements, we are required to pay all registration expenses, including registration fees, printing expenses, fees and disbursements of our counsel and accountants and reasonable fees and disbursements not to exceed \$50,000 of one counsel representing the selling stockholders, other than any underwriting discounts and commissions, related to any demand or incidental registration.

The Registration Rights Agreements contain customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

### ***Preferred Stock***

Our board of directors has the authority, without further action by the stockholders, to issue up to 5,500,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, redemption rights, liquidation preferences, sinking fund terms and the number of shares constituting any series or the designation of such series, any or all of which may be greater than the rights of common stock. The issuance of preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing change in our control or other corporate action. Upon closing of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

### ***Anti-Takeover Provisions***

Our certificate of incorporation and bylaws will contain certain provisions that are intended to enhance the likelihood of continuity and stability in the composition of our board of directors but which may have the effect of delaying, deferring or preventing a future takeover or change in control of us unless such takeover or change in control is approved by our board of directors.

### ***Staggered Board; Removal of Directors***

Our amended and restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing of the offering divide our board of directors into three classes with staggered three-year terms. In addition, our certificate of incorporation and our bylaws to be effective upon the closing of the offering provide that directors may be removed only for cause and only by the affirmative vote of the holders of 66<sup>2</sup>/<sub>3</sub>% of our shares of capital stock present in person or by proxy and entitled to vote. Under our certificate of incorporation and bylaws to be effective upon the closing

of the offering, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office, although less than a quorum, or by a sole remaining director and shall not be filled by the stockholders. Furthermore, our amended and restated certificate of incorporation to be effective upon the closing of the offering provides that the authorized number of directors may be changed only by the resolution of our board of directors. The classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

***Stockholder Action; Special Meeting of Stockholders; Advance Notice Requirements for Stockholder Proposals and Director Nominations***

Our amended and restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing of the offering provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our certificate of incorporation and our bylaws to be effective upon the closing of the offering also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by the chairman of our board of directors, our chief executive officer or our board of directors. In addition, our bylaws to be effective upon the closing of the offering establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors, or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities. These provisions also could discourage a third party from making a tender offer for our common stock because even if the third party acquired a majority of our outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

***Super-Majority Voting***

The DGCL provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws to be effective upon the closing of the offering may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 66<sup>2</sup>/<sub>3</sub>% of the votes that all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 66<sup>2</sup>/<sub>3</sub>% of the votes that all our stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above.

***Section 203 of the Delaware General Corporation Law***

Upon completion of this offering, we will be subject to the provisions of Section 203 of the DGCL. In general, Section 203 prohibits a publicly-held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a

prescribed manner. A "business combination" includes, among other things, a merger, asset or stock sale or other transaction resulting in a financial benefit to the interested stockholder. An "interested stockholder" is a person who, together with affiliates and associates, owns, or did own within three years prior to the determination of interested stockholder status, 15% or more of the corporation's voting stock.

Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions: before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder; upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances; or at or after the time the stockholder became interested, the business combination was approved by our board of directors of the corporation and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

#### **Exclusive Forum Selection**

Our certificate of incorporation to be effective upon the closing of the offering provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of our company, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware, or (4) any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine. Although our certificate of incorporation contains the choice of forum provision described above, it is possible that a court could rule that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

#### **Authorized but Unissued Shares**

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing requirements of the Nasdaq Global Market. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

#### **Transfer Agent and Registrar**

The transfer agent and registrar for our common stock is . The transfer agent and registrar's address is .

#### **Nasdaq Global Market**

We plan to apply to have our common stock listed on the Nasdaq Global Market under the symbol "YMAB."

## SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock, and an active trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options or in the public market after this offering, or the anticipation of these sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of our equity securities.

Based upon the \_\_\_\_\_ shares of our common stock that were outstanding on \_\_\_\_\_, 2018, upon the closing of this offering, we will have \_\_\_\_\_ outstanding \_\_\_\_\_ shares of our common stock, after giving effect to the issuance of \_\_\_\_\_ shares of our common stock in this offering and assuming no exercise by the underwriters of their option to purchase additional shares and no exercise of options outstanding as of December 31, 2017.

Of the shares to be outstanding immediately after the closing of this offering, we expect that the \_\_\_\_\_ shares to be sold in this offering, assuming that the underwriters do not exercise their option to purchase additional shares, will be freely tradable without restriction or further registration under the Securities Act unless purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act, or Rule 144, whose sales would be subject to the Rule 144 resale restrictions described below, other than the holding period requirement.

The remaining \_\_\_\_\_ shares of our common stock and any shares purchased in this offering by our affiliates will be "restricted securities" under Rule 144, and we expect that substantially all of these restricted securities will be subject to the 180-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

### Rule 144

In general, under Rule 144, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months may sell any unrestricted securities, as well as restricted securities that the person has beneficially owned for at least six months, including the holding period of any prior owner other than one of our affiliates, under Rule 144. Affiliates selling restricted or unrestricted securities may sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately \_\_\_\_\_ shares immediately after this offering; and
- the average weekly trading volume of our common stock on the Nasdaq Global Market during the four calendar weeks preceding the filing of a Notice of Proposed Sale of Securities Pursuant to Rule 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us.

Upon expiration of the 180-day lock-up period described below, approximately \_\_\_\_\_ shares of our common stock will be eligible for sale under Rule 144, including shares eligible for resale immediately upon the closing of this offering as described above. We cannot estimate the number of shares of our common stock that our existing stockholders will elect to sell under Rule 144.

### **Rule 701**

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell these shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the holding period requirements of Rule 144 and without regard to the volume of such sales or the availability of public information about us. Subject to the 180-day lock-up period described below, approximately \_\_\_\_\_ shares of our common stock will be eligible for sale in accordance with Rule 701.

### **Lock-Up Agreements**

We, each of our executive officers and directors and the holders of our outstanding stock have agreed that, without the prior written consent of Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC, on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus:

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant for the sale of, or otherwise dispose of or transfer any shares of our common stock or any securities convertible into or exchangeable or exercisable for our common stock;
- exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock, or with respect to the filing of any registration statement in connection therewith under the Securities Act; or
- enter into any swap or any other agreement or any transaction that transfers, in whole or in part, the economic consequence of ownership of our common stock, whether any transaction described above is to be settled by delivery of our common stock or such other securities, in cash or otherwise.

### **Stock Options**

As of \_\_\_\_\_, 2018, we had outstanding options to purchase \_\_\_\_\_ shares of our common stock, of which options to purchase \_\_\_\_\_ shares were vested and exercisable. Following this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding options under the 2015 Plan and options and other awards issuable pursuant to the 2015 Plan. Shares covered by these registration statements will then be eligible for sale in the public markets, subject to vesting restrictions, any applicable lock-up agreements described above and Rule 144 limitations applicable to affiliates.

### **Registration Rights**

After the completion of this offering, the holders of up to \_\_\_\_\_ shares of our common stock will be entitled to certain rights with respect to the registration of such shares under the Securities Act. The registration of these shares of our common stock under the Securities Act would



result in these shares becoming eligible for sale in the public market without restriction under the Securities Act immediately upon the effectiveness of such registration, subject to the Rule 144 limitations applicable to affiliates. See the section titled "Description of Capital Stock—Registration Rights" for a description of these registration rights.

### **Registration Statement**

In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act to register all of the shares of our common stock subject to equity awards outstanding or reserved for issuance under our equity compensation plans. The shares of our common stock covered by such registration statements will be eligible for sale in the public market without restriction under the Securities Act immediately upon the effectiveness of such registration statements, subject to vesting restrictions, the conditions of Rule 144 applicable to affiliates and any applicable market standoff agreements and lock-up agreements. See the section titled "Executive and Director Compensation—Stock Option and Other Compensation Plans" for a description of our equity compensation plans.

**MATERIAL U.S. FEDERAL INCOME AND ESTATE TAX CONSIDERATIONS  
FOR NON-U.S. HOLDERS OF COMMON STOCK**

The following is a discussion of material U.S. federal income and estate tax considerations relating to the ownership and disposition of our common stock by a non-U.S. holder. For purposes of this discussion, the term "non-U.S. holder" means a beneficial owner (other than a partnership or other entity or arrangement that is treated as a partnership for U.S. federal income tax purposes) of our common stock that is not, for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation, or other entity treated as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States, any state thereof, or the District of Columbia;
- an estate the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust if (1) a U.S. court is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have authority to control all substantial decisions of the trust or (2) the trust has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a U.S. person.

This discussion does not address the tax treatment of partnerships or other entities or arrangements that are pass-through entities for U.S. federal income tax purposes or persons who hold their common stock through partnerships or other such pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult that partner's tax advisor regarding the tax consequences of the ownership and disposition of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the Code, final, temporary and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and all of which are subject to change or to differing interpretation, possibly with retroactive effect. In particular, Congress currently is considering significant tax reform proposals to the U.S. federal tax laws. Any change could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, or the IRS, will not challenge one or more of the tax consequences described in this prospectus.

We assume in this discussion that each non-U.S. holder holds shares of our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all aspects of U.S. federal income and estate taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of U.S. state, local or non-U.S. taxes, the alternative minimum tax, or the Medicare tax on net investment income. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- financial institutions;
- brokers, dealers or traders in securities;
- tax-exempt organizations;
- pension plans;

- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment or who have elected to mark securities to market;
- non-U.S. holders who hold more than 5% of our common stock, directly or by attribution;
- insurance companies;
- controlled foreign corporations;
- passive foreign investment companies;
- companies that accumulate earnings to avoid U.S. federal income tax;
- government organizations; and
- certain U.S. expatriates.

**THIS DISCUSSION IS FOR INFORMATION ONLY AND IS NOT, AND IS NOT INTENDED TO BE, LEGAL OR TAX ADVICE. PROSPECTIVE INVESTORS SHOULD CONSULT THEIR TAX ADVISORS REGARDING THE U.S. FEDERAL, STATE, LOCAL, ESTATE AND NON-U.S. INCOME AND OTHER TAX CONSIDERATIONS, INCLUDING THAT OF INCOME TAX TREATIES, OF ACQUIRING, HOLDING AND DISPOSING OF OUR COMMON STOCK.**

### **Distributions**

As discussed under "Dividend Policy" above, we do not expect to make cash dividends to holders of our common stock in the foreseeable future. If we make distributions in respect of our common stock, those distributions generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles, subject to the tax treatment described in this section. If a distribution exceeds our current and accumulated earnings and profits, the excess amount of the distribution will be treated as a tax-free return of the non-U.S. holder's investment, up to the holder's tax basis in the common stock and correspondingly reduce (but not below zero) the non-U.S. holder's basis in such stock. Any remaining excess of the distribution once the non-U.S. holder's basis is reduced to zero will be treated as capital gain, subject to the tax treatment described below under the heading "Gain on Sale, Exchange or Other Taxable Disposition of Our Common Stock." Any such distributions will also be subject to the discussions below under the headings "Information Reporting and Backup Withholding" and "FATCA."

Subject to the discussion below on effectively connected income, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States, and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, generally are exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements (generally including provision of a valid IRS Form W-8ECI (or applicable successor form) certifying that the dividends are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States). However, such U.S. effectively connected income, net of specified deductions and credits, is taxed in the hands of the non-U.S. holder at the same graduated U.S. federal income tax rates as would apply if such holder were a U.S. person (as defined in the Code). Any income effectively connected with a trade or business conducted within the United States that is received by a non-U.S. holder that is classified as a

corporation for U.S. federal income tax purposes may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty and the specific methods available to them to satisfy such requirements.

A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

#### **Gain on Sale, Exchange or Other Taxable Disposition of Our Common Stock**

Subject to the discussions below under the headings "Information Reporting and Backup Withholding" and "FATCA," a non-U.S. holder generally will not be subject to U.S. federal income tax or withholding tax on any gain realized upon such non-U.S. holder's sale, exchange or other disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the United States, and, if an applicable income tax treaty so provides, the gain is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the United States; in these cases, the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code), and, if the non-U.S. holder is a non-U.S. corporation, an additional branch profits tax at a rate of 30% (or a lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) may also apply;
- the non-U.S. holder is a non-resident alien present in the United States for 183 days or more in the taxable year of the disposition and certain other requirements are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S.-source capital losses of the non-U.S. holder, if any; or
- we are or have been, at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation" unless our common stock is regularly traded on an established securities market and the non-U.S. holder held no more than 5% of our outstanding common stock, directly, indirectly or by attribution, during the shorter of the five-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a "U.S. real property holding corporation" if the fair market value of its "U.S. real property interests" (as defined in the Code and applicable regulations) equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we believe that we are not currently, and we do not anticipate becoming, a "U.S. real property holding corporation" for U.S. federal income tax purposes. No assurance can be *provided* that our common stock will be regularly traded on an established securities market for purposes of the rule described above.

Non-U.S. holders are urged to consult with their tax advisors on the treatment of any gain on the disposition of our common stock based on their particular circumstances.

### **Information Reporting and Backup Withholding**

The gross amount of the distributions on our common stock paid to each non-U.S. holder and the tax withheld, if any, with respect to such distributions must be reported annually to the IRS and to such holder. Non-U.S. holders generally will have to comply with specific certification procedures to establish that the holder is not a U.S. person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Generally, a holder will comply with such procedures if it provides a properly executed IRS Form W-8BEN or W-8BEN-E (or other applicable Form W-8), or otherwise establishes an exemption through an income tax treaty or otherwise. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above under "Distributions," generally will be exempt from U.S. backup withholding if such forms described herein are timely and properly provided. Notwithstanding the foregoing, backup withholding may apply if we have actual knowledge, or reason to know, that the non-U.S. holder is a U.S. person (as defined in the Code) that is not an exempt recipient.

Information reporting and backup withholding generally will apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or non-U.S., unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, *provided* that an appropriate claim is timely filed with the IRS.

### **FATCA**

Provisions of the Code commonly known as the Foreign Account Tax Compliance Act, or FATCA, generally impose a 30% withholding tax on dividends on, and gross proceeds from the sale or disposition of, our common stock if paid to a foreign entity unless: (i) if the foreign entity is a "foreign financial institution," (as defined in the Code), the foreign entity undertakes certain due diligence, information reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," and the foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA.

Withholding under FATCA generally (1) applies to payments of dividends on our common stock and (2) will apply to payments of gross proceeds from a sale or other disposition of our common stock made after December 31, 2018. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this section. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of the tax. Non-U.S. holders

should consult their tax advisors regarding the possible implications of FATCA on their investment in our common stock.

### **Federal Estate Tax**

Shares of our common stock that are owned or treated as owned by an individual who is not a citizen or resident of the United States (as specially defined for U.S. federal estate tax purposes) at the time of death are considered U.S. situs assets and will be included in the individual's gross estate for U.S. federal estate tax purposes. Such shares, therefore, may be subject to U.S. federal estate tax, unless an applicable estate tax or other treaty provides otherwise.

**The preceding discussion of material U.S. federal tax considerations is for information only. It is not legal or tax advice. Prospective investors should consult their tax advisors regarding the particular U.S. federal, state, local and non-U.S. tax consequences of purchasing, holding and disposing of our common stock, including the consequences of any proposed changes in applicable laws.**

## UNDERWRITING

Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC are acting as representatives of each of the underwriters named below. Subject to the terms and conditions set forth in an underwriting agreement among us and the underwriters, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the number of shares of common stock set forth opposite its name below.

<u>Underwriter</u>	<u>Number of Shares</u>
Merrill Lynch, Pierce, Fenner & Smith Incorporated	
Cowen and Company, LLC	
Total	<u><u>          </u></u>

Subject to the terms and conditions set forth in the underwriting agreement, the underwriters have agreed, severally and not jointly, to purchase all of the shares sold under the underwriting agreement if any of these shares are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the nondefaulting underwriters may be increased or the underwriting agreement may be terminated.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, or to contribute to payments the underwriters may be required to make in respect of those liabilities.

The underwriters are offering the shares, subject to prior sale, when, as and if issued to and accepted by them, subject to approval of legal matters by their counsel, including the validity of the shares, and other conditions contained in the underwriting agreement, such as the receipt by the underwriters of officer's certificates and legal opinions. The underwriters reserve the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

### Commissions and Discounts

The representatives have advised us that the underwriters propose initially to offer the shares to the public at the public offering price set forth on the cover page of this prospectus and to dealers at that price less a concession not in excess of \$        per share. After the initial offering, the public offering price, concession or any other term of the offering may be changed.

The following table shows the public offering price, underwriting discount and proceeds before expenses to us. The information assumes either no exercise or full exercise by the underwriters of their option to purchase additional shares.

	<u>Per Share</u>	<u>Without Option</u>	<u>With Option</u>
Public offering price	\$	\$	\$
Underwriting discount	\$	\$	\$
Proceeds, before expenses, to us	\$	\$	\$

The expenses of the offering, not including the underwriting discount, are estimated at \$        and are payable by us. We have also agreed to reimburse the underwriters for their expenses relating to clearance of this offering with the Financial Industry Regulatory Authority in an amount up to \$60,000.

## Option to Purchase Additional Shares

We have granted an option to the underwriters, exercisable for 30 days after the date of this prospectus, to purchase up to \_\_\_\_\_ additional shares at the public offering price, less the underwriting discount. If the underwriters exercise this option, each will be obligated, subject to conditions contained in the underwriting agreement, to purchase a number of additional shares proportionate to that underwriter's initial amount reflected in the above table.

## No Sales of Similar Securities

We, our executive officers and directors and our other existing security holders have agreed not to sell or transfer any common stock or securities convertible into, exchangeable for, exercisable for, or repayable with common stock, for 180 days after the date of this prospectus without first obtaining the written consent of Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC. Specifically, we and these other persons have agreed, with certain limited exceptions, not to directly or indirectly

- offer, pledge, sell or contract to sell any common stock,
- sell any option or contract to purchase any common stock,
- purchase any option or contract to sell any common stock,
- grant any option, right or warrant for the sale of any common stock,
- lend or otherwise dispose of or transfer any common stock,
- request or demand that we file a registration statement or make a confidential submission related to the common stock, or
- enter into any swap or other agreement that transfers, in whole or in part, the economic consequence of ownership of any common stock whether any such swap or transaction is to be settled by delivery of shares or other securities, in cash or otherwise.

This lock-up provision applies to common stock and to securities convertible into or exchangeable or exercisable for or repayable with common stock. It also applies to common stock owned now or acquired later by the person executing the agreement or for which the person executing the agreement later acquires the power of disposition. Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC, in their sole discretion, may release the common stock and other securities subject to the lock-up agreements described above in whole or in part at any time with or without notice. In addition, in the event that any stockholder holding in excess of 5% of our outstanding shares, or a Major Holder, is granted an early release from the lock-up restrictions with respect to our securities in an aggregate amount in excess of one percent of our issued and outstanding shares (whether in one or multiple releases), then each other Major Holder automatically will be granted an equivalent early release from its obligations under the lock-up agreement on a pro-rata basis. Such release shall not be applicable in the event of an underwritten primary or secondary public offering or sale of our common stock during the period ending 180 days after the date of this prospectus.

## Nasdaq Global Market Listing

We expect the shares to be approved for listing on the Nasdaq Global Market, subject to notice of issuance, under the symbol "YMAB."



## **Determination of Estimated Offering Price**

Before this offering, there has been no public market for our common stock. The initial public offering price will be determined through negotiations between us and the representatives. In addition to prevailing market conditions, the factors to be considered in determining the initial public offering price are:

- the valuation multiples of publicly traded companies that the representatives believe to be comparable to us,
- our financial information,
- the history of, and the prospects for, our company and the industry in which we compete,
- an assessment of our management, its past and present operations, and the prospects for, and timing of, our future revenues,
- the present state of our development, and
- the above factors in relation to market values and various valuation measures of other companies engaged in activities similar to ours.

An active trading market for the shares may not develop. It is also possible that after the offering the shares will not trade in the public market at or above the initial public offering price.

The underwriters do not expect to sell more than 5% of the shares in the aggregate to accounts over which they exercise discretionary authority.

## **Price Stabilization, Short Positions and Penalty Bids**

Until the distribution of the shares is completed, SEC rules may limit underwriters and selling group members from bidding for and purchasing our common stock. However, the representatives may engage in transactions that stabilize the price of the common stock, such as bids or purchases to peg, fix or maintain that price.

In connection with the offering, the underwriters may purchase and sell our common stock in the open market. These transactions may include short sales, purchases on the open market to cover positions created by short sales and stabilizing transactions. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering. "Covered" short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares described above. The underwriters may close out any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the option granted to them. "Naked" short sales are sales in excess of such option. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of our common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of shares of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Similar to other purchase transactions, the underwriters' purchases to cover the syndicate short sales may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock may be higher than the price that might otherwise exist in the open market. The underwriters may conduct these transactions on the Nasdaq Global Market, in the over-the-counter market or otherwise.

Neither we nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. In addition, neither we nor any of the underwriters make any representation that the representatives will engage in these transactions or that these transactions, once commenced, will not be discontinued without notice.

### **Electronic Distribution**

In connection with the offering, certain of the underwriters or securities dealers may distribute prospectuses by electronic means, such as e-mail.

### **Other Relationships**

Some of the underwriters and their affiliates have engaged in, and may in the future engage in, investment banking and other commercial dealings in the ordinary course of business with us or our affiliates. They have received, or may in the future receive, customary fees and commissions for these transactions.

In addition, in the ordinary course of their business activities, the underwriters and their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers. Such investments and securities activities may involve securities and/or instruments of ours or our affiliates. The underwriters and their affiliates may also make investment recommendations and/or publish or express independent research views in respect of such securities or financial instruments and may hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

### **Notice to Prospective Investors in the European Economic Area**

In relation to each member state of the European Economic Area, no offer of shares which are the subject of the offering has been, or will be made to the public in that Member State, other than under the following exemptions under the Prospectus Directive:

- (a) to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- (b) to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), subject to obtaining the prior consent of the Representatives for any such offer; or
- (c) in any other circumstances falling within Article 3(2) of the Prospectus Directive,

*provided* that no such offer of shares referred to in (a) to (c) above shall result in a requirement for us or any representative to publish a prospectus pursuant to Article 3 of the Prospectus Directive, or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

Each person located in a Member State to whom any offer of shares is made or who receives any communication in respect of an offer of shares, or who initially acquires any shares will be deemed to have represented, warranted, acknowledged and agreed to and with each representative and us that (1) it is a "qualified investor" within the meaning of the law in that Member State implementing

Article 2(1)(e) of the Prospectus Directive; and (2) in the case of any shares acquired by it as a financial intermediary as that term is used in Article 3(2) of the Prospectus Directive, the shares acquired by it in the offer have not been acquired on behalf of, nor have they been acquired with a view to their offer or resale to, persons in any Member State other than qualified investors, as that term is defined in the Prospectus Directive, or in circumstances in which the prior consent of the Representatives has been given to the offer or resale; or where shares have been acquired by it on behalf of persons in any Member State other than qualified investors, the offer of those shares to it is not treated under the Prospectus Directive as having been made to such persons.

We, the representatives and their respective affiliates will rely upon the truth and accuracy of the foregoing representations, acknowledgments and agreements.

This prospectus has been prepared on the basis that any offer of shares of our common stock in any Member State will be made pursuant to an exemption under the Prospectus Directive from the requirement to publish a prospectus for offers of shares. Accordingly any person making or intending to make an offer in that Member State of shares which are the subject of the offering contemplated in this prospectus may only do so in circumstances in which no obligation arises for us or any of the representatives to publish a prospectus pursuant to Article 3 of the Prospectus Directive in relation to such offer. Neither we nor the representatives have authorized, nor do they authorize, the making of any offer of shares in circumstances in which an obligation arises for us or the representatives to publish a prospectus for such offer.

For the purposes of this provision, the expression an "offer of shares to the public" in relation to any shares in any Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase or subscribe the shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (as amended) and includes any relevant implementing measure in each Member State.

The above selling restriction is in addition to any other selling restrictions set out below.

#### **Notice to Prospective Investors in the United Kingdom**

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Directive) (i) who have professional experience in matters relating to investments falling within Article 19 (5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended, or the Order, and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). This document must not be acted on or relied on in the United Kingdom by persons who are not relevant persons. In the United Kingdom, any investment or investment activity to which this document relates is only available to, and will be engaged in with, relevant persons.

#### **Notice to Prospective Investors in Switzerland**

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing

material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, our company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

#### **Notice to Prospective Investors in the Dubai International Financial Centre**

This prospectus relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or DFSA. This prospectus is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth herein and has no responsibility for the prospectus. The shares to which this prospectus relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus you should consult an authorized financial advisor.

#### **Notice to Prospective Investors in Australia**

No placement document, prospectus, product disclosure statement or other disclosure document has been lodged with the Australian Securities and Investments Commission ("ASIC"), in relation to the offering. This prospectus does not constitute a prospectus, product disclosure statement or other disclosure document under the Corporations Act 2001 (the "Corporations Act"), and does not purport to include the information required for a prospectus, product disclosure statement or other disclosure document under the Corporations Act.

Any offer in Australia of the shares may only be made to persons (the "Exempt Investors") who are "sophisticated investors" (within the meaning of section 708(8) of the Corporations Act), "professional investors" (within the meaning of section 708(11) of the Corporations Act) or otherwise pursuant to one or more exemptions contained in section 708 of the Corporations Act so that it is lawful to offer the shares without disclosure to investors under Chapter 6D of the Corporations Act.

The shares applied for by Exempt Investors in Australia must not be offered for sale in Australia in the period of 12 months after the date of allotment under the offering, except in circumstances where disclosure to investors under Chapter 6D of the Corporations Act would not be required pursuant to an exemption under section 708 of the Corporations Act or otherwise or where the offer is pursuant to a disclosure document which complies with Chapter 6D of the Corporations Act. Any person acquiring shares must observe such Australian on-sale restrictions.

This prospectus contains general information only and does not take account of the investment objectives, financial situation or particular needs of any particular person. It does not contain any securities recommendations or financial product advice. Before making an investment decision, investors need to consider whether the information in this prospectus is appropriate to their needs, objectives and circumstances, and, if necessary, seek expert advice on those matters.

### **Notice to Prospective Investors in Hong Kong**

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance; or (b) in other circumstances which do not result in the document being a "prospectus" as defined in the Companies Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of that Ordinance. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" as defined in the Securities and Futures Ordinance and any rules made under that Ordinance.

### **Notice to Prospective Investors in Japan**

The shares have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948, as amended) and, accordingly, will not be offered or sold, directly or indirectly, in Japan, or for the benefit of any Japanese Person or to others for re-offering or resale, directly or indirectly, in Japan or to any Japanese Person, except in compliance with all applicable laws, regulations and ministerial guidelines promulgated by relevant Japanese governmental or regulatory authorities in effect at the relevant time. For the purposes of this paragraph, "Japanese Person" shall mean any person resident in Japan, including any corporation or other entity organized under the laws of Japan.

### **Notice to Prospective Investors in Singapore**

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA"), (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275, of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- (a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or
- (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities (as defined in Section 239(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after

that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

- (a) to an institutional investor or to a relevant person defined in Section 275(2) of the SFA, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;
- (b) where no consideration is or will be given for the transfer;
- (c) where the transfer is by operation of law;
- (d) as specified in Section 276(7) of the SFA; or
- (e) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore.

#### **Notice to Prospective Investors in Canada**

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, *provided* that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

## LEGAL MATTERS

The validity of the shares of our common stock offered hereby is being passed upon for us by Satterlee Stephens LLP. Certain legal matters relating to this offering will be passed upon for the underwriters by Shearman & Sterling LLP.

## EXPERTS

The financial statements as of December 31, 2016 and for the year then ended included in this prospectus have been so included in reliance on the report of PricewaterhouseCoopers LLP, an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

### *Change in our public accounting firm*

On October 19, 2017, we dismissed PricewaterhouseCoopers Statsautoriseret Revisionspartnerselskab, based in Copenhagen, Denmark, or PwC DK, as our independent accountants. The decision to dismiss PwC DK as our independent registered public accounting firm was approved by our board of directors.

The reports of PwC DK on our 2016 and 2015 consolidated financial statements did not contain any adverse opinion or disclaimer of opinion, nor was such report qualified or modified as to uncertainty, audit scope or accounting principles. During the year ended December 31, 2016 and 2015 and through the subsequent interim period through October 19, 2017, there were no disagreements between us and PwC DK on any matter of accounting principles or practices, financial statement disclosure or auditing scope or procedure, which disagreement, if not resolved to the satisfaction of PwC DK, would have caused them to make reference to the subject matter of the disagreement in connection with their reports on the financial statements for such years. During the years ended December 31, 2016 and 2015 and the subsequent interim period through October 19, 2017, there have been no reportable events (as defined in S-K 304(a)(1)(v)).

On October 20, 2017, we engaged PricewaterhouseCoopers LLP, or PwC, as our independent registered public accounting firm, to audit our consolidated financial statements as of and for the year ended December 31, 2016.

During our year ended December 31, 2016 and in the subsequent interim period through October 20, 2017, neither we nor anyone on our behalf consulted with PwC regarding either: (i) the application of accounting principles to a specified transaction, either completed or proposed, or the type of audit opinion that might be rendered on our financial statements, and neither a written report was provided to us or oral advice was provided to us that PwC concluded was an important factor considered by us in reaching a decision as to the accounting, auditing or financial reporting issue; or (ii) any matter that was either the subject of a disagreement or reportable event as defined in Regulation S-K, Item 304(a)(1)(iv) and Item 304(a)(1)(v), respectively.

We delivered a copy of this disclosure to PwC DK and requested that they furnish us a letter addressed to the SEC stating whether they agree with the above statements. In their letter to the SEC dated \_\_\_\_\_, attached as Exhibit 16.1 to the registration statement of which this prospectus forms a part, PwC DK states that they agree with the statements above concerning their firm.

## WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of our common stock we are offering to sell. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and amendments to the registration statement. Some items are omitted in accordance with the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits, schedules and amendments to the registration statement. Statements contained in this prospectus about the contents of any contract, agreement or other document filed as an exhibit are not necessarily complete, and, in each instance, we refer you to the copy of the contract, agreement or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You may read and copy the registration statement of which this prospectus is a part at the SEC's public reference room, which is located at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. You can request copies of the registration statement by writing to the Securities and Exchange Commission and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the SEC's public reference room. In addition, the SEC maintains an Internet website, which is located at [www.sec.gov](http://www.sec.gov), that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the SEC's Internet website.

Upon completion of this offering, we will be subject to the information reporting requirements of the Exchange Act, and we will file annual, quarterly and proxy reports, proxy statements and other information with the SEC. We intend to furnish our stockholders with annual reports containing financial statements certified by an independent registered public accounting firm. We also maintain a website at [www.ymabs.com](http://www.ymabs.com). The information contained on, or that can be accessed through, our website is not a part of, and is not incorporated into, this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.



**Y-MABS THERAPEUTICS, INC.**  
**INDEX TO CONSOLIDATED FINANCIAL STATEMENTS**  
**FOR THE YEAR ENDED DECEMBER 31, 2016**

	<u>Page</u>
<a href="#">Report of Independent Registered Public Accounting Firm</a>	<a href="#">F-2</a>
<a href="#">Consolidated Balance Sheet</a>	<a href="#">F-3</a>
<a href="#">Consolidated Statement of Comprehensive Loss</a>	<a href="#">F-4</a>
<a href="#">Consolidated Statement of Changes in Stockholders' Equity For the Year Ended December 31, 2016</a>	<a href="#">F-5</a>
<a href="#">Consolidated Statement of Cash Flows</a>	<a href="#">F-6</a>
<a href="#">Notes to Consolidated Financial Statements</a>	<a href="#">F-7</a>

**Report of Independent Registered Public Accounting Firm**

To the Board of Directors and Shareholders of Y-mAbs Therapeutics, Inc.:

In our opinion, the accompanying consolidated balance sheet and the related consolidated statements of comprehensive loss, changes in stockholders' equity and cash flows present fairly, in all material respects, the financial position of Y-mAbs Therapeutics, Inc. and its subsidiary as of December 31, 2016, and the results of their operations and their cash flows for the year then ended in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit. We conducted our audit of these statements in accordance with the standards of the Public Company Accounting Oversight Board (United States) and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

Florham Park, New Jersey  
February 13, 2018

**Y-MABS THERAPEUTICS, INC.****Consolidated Balance Sheet****(in thousands, except share data)**

	<u>December 31,</u> <u>2016</u>
<b>ASSETS</b>	
<b>CURRENT ASSETS</b>	
Cash and cash equivalents	\$ 16,875
Restricted cash	28
Other current assets	358
Total current assets	<u>17,261</u>
<b>TOTAL ASSETS</b>	<u>\$ 17,261</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>	
<b>LIABILITIES</b>	
Accounts payable	\$ 2,227
Accrued liabilities	748
Total current liabilities	2,975
Accrued long-term liabilities	2,225
<b>TOTAL LIABILITIES</b>	<u>5,200</u>
<b>STOCKHOLDERS' EQUITY</b>	
Preferred stock \$0.0001 par value, 1,000,000 shares authorized at December 31, 2016; None issued at December 31, 2016	—
Common stock, \$0.0001 par value, 50,000,000 shares authorized at December 31, 2016; 16,552,884 shares issued at December 31, 2016	2
Additional paid in capital	34,429
Accumulated other comprehensive income	30
Accumulated deficit	(22,400)
<b>TOTAL STOCKHOLDERS' EQUITY</b>	<u>12,061</u>
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<u>\$ 17,261</u>

The accompanying notes are an integral part of the consolidated financial statements

**Y-MABS THERAPEUTICS, INC.****Consolidated Statement of Comprehensive Loss****(In thousands, except share and per share data)**

	<b>For The Year Ended December 31, 2016</b>
<b>OPERATING EXPENSES</b>	
Research and development	\$ 13,855
General and administrative	3,184
Total operating expenses	<u>17,039</u>
<b>OTHER EXPENSES</b>	
Other expenses	18
<b>NET LOSS</b>	<u>\$ (17,057)</u>
Other comprehensive loss	
Foreign currency translation	40
<b>COMPREHENSIVE LOSS</b>	<u>\$ (17,017)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (1.21)</u>
Weighted average common shares outstanding, basic and diluted	<u>14,087,456</u>

The accompanying notes are an integral part of the consolidated financial statements

## Y-MABS THERAPEUTICS, INC.

## Consolidated Statement of Changes in Stockholders' Equity

For the Year Ended December 31, 2016

(In thousands, except share data)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive (Loss)/Income	Accumulated Deficit	Stockholders' Equity
	Shares	Amount				
Balance January 1, 2016	11,243,384	\$ 1	\$ 12,920	\$ (10)	\$ (5,343)	\$ 7,568
Issuance of common stock to investors, net of issuance costs	3,945,171	1	19,337	—	—	19,338
Issuance of common stock to strategic partner	999,929	—	2,280	—	—	2,280
Issuance of common stock to nonemployees	448,000	—	—	—	—	—
Repurchase of common stock	(83,600)	—	(366)	—	—	(366)
Stock-based compensation expense	—	—	258	—	—	258
Other comprehensive income	—	—	—	40	—	40
Net loss	—	—	—	—	(17,057)	(17,057)
Balance December 31, 2016	16,552,884	\$ 2	\$ 34,429	\$ 30	\$ (22,400)	\$ 12,061

The accompanying notes are an integral part of the consolidated financial statements

**Y-MABS THERAPEUTICS, INC.**  
**Consolidated Statement of Cash Flows**  
**(In thousands)**

	<u>December 31,</u> <u>2016</u>
<b>CASH FLOWS FROM OPERATING ACTIVITIES</b>	
Net loss	\$ (17,057)
Adjustments to reconcile net loss to net cash used in operating activities:	
Stock-based compensation	258
Non-cash expense in connection with equity issuance to strategic partner	2,280
Changes in assets and liabilities:	
Other current assets	(309)
Accounts payable	836
Accrued liabilities and other	2,826
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<u>(11,166)</u>
<b>CASH FLOWS FROM FINANCING ACTIVITIES</b>	
Proceeds from issuance of common stock, net of issuance costs	19,338
Repurchase of common stock	(366)
<b>NET CASH PROVIDED BY FINANCING ACTIVITIES</b>	<u>18,972</u>
<b>NET INCREASE IN CASH AND CASH EQUIVALENTS</b>	7,806
Cash and cash equivalents at the beginning of year	9,069
Cash and cash equivalents at the end of year	<u>\$ 16,875</u>

The accompanying notes are an integral part of the consolidated financial statements

**Y-MABS THERAPEUTICS, INC.**

**Notes to Consolidated Financial Statements**

**December 31, 2016**

**NOTE 1—ORGANIZATION AND DESCRIPTION OF BUSINESS**

Y-mAbs Therapeutics, Inc. ("we," "us," "our," the "Company," or "YmAbs") is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel antibody therapeutic products to treat cancer.

We have entered into a worldwide license and research agreement (the "MSK License") with Memorial Sloan-Kettering Cancer Center ("MSK") our strategic partner, under which we have obtained the exclusive rights to two clinical stage antibody-based product development programs for the treatment of neuroblastoma and other pediatric oncology indications. The MSK License also includes a protein Multimerization Platform Technology—MULTI TAG™, and an option to obtain the rights to certain chimeric antigen receptor T-cell, or CAR-T, technologies, as well as rights to next-generation humanized, affinity matured bispecific antibodies.

The Company is headquartered in New York and was incorporated on April 30, 2015 under the laws of the State of Delaware.

**NOTE 2—BASIS OF PRESENTATION**

The Company has not generated any revenue and has incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development; technological uncertainty; uncertainty regarding patents and proprietary rights; uncertainty in obtaining FDA approval in the United States and regulatory approval in other jurisdictions; marketing or sales capability or experience; uncertainty in getting adequate payor coverage and reimbursement; and dependence on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities.

The Company's drug candidates are in the development stage. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

The Company's financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has experienced negative cash flows and had an accumulated deficit of \$22,400,000 as of December 31, 2016. Through December 31, 2016, the Company has funded its operations through proceeds from sales of shares of its common stock. As of December 31, 2016, the Company had cash and cash equivalents of \$16,875,000. During the first quarter of 2017, the Company completed a non-public offering of its common stock at \$8.50 per share to investors. The non-public offering was initiated in December 2016, and the Company raised a total of \$14,559,000, of which \$4,421,000 was received in December 2016 and \$10,138,000 was received in January and February 2017. In October

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 2—BASIS OF PRESENTATION (Continued)**

and November 2017, the Company raised a total of \$80,000,000 in an additional non-public offering of common stock of at \$9.35 per share to investors. As of February 13, 2018, the issuance date of these financial statements, the Company expects that its cash and cash equivalents at December 31, 2016 combined with funds raised subsequent to the balance sheet date will be sufficient to fund its operating expenses and capital expenditure requirements through at least the next twelve months. The future viability of the Company, until such time that the Company has commercialized any of its products, is dependent on its ability to raise additional capital to finance its operations. The Company's failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

The Company is seeking to complete an initial public offering of its common shares. In the event the Company does not complete an initial public offering, and fails to generate cash from its operating activities, the Company expects to seek additional funding through private equity financings, debt financings, or other capital sources, including collaborations with other companies or other strategic transactions. The Company may not be able to obtain financing on acceptable terms, or at all. The terms of any financing may adversely affect the holdings or the rights of the Company's shareholders. The Company may not be able to obtain additional future financing on acceptable terms, or at all, and the Company may not be able to enter into collaboration arrangements or obtain government grants. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain funding, the Company could be forced to delay, reduce or eliminate its research and development programs, product portfolio expansion or commercialization efforts, which could adversely affect its business prospects.

The accompanying consolidated financial statements reflected the accounts of the Company and its wholly-owned subsidiary and have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). All intercompany balances and transactions have been eliminated.

**NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES*****Use of Estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these financial statements include, but are not limited to, the accrual for research and development expenses, the accrual of milestone and royalty payments, the valuation of shares of common stock and stock options. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

***Cash and Cash Equivalents***

The Company considers all highly-liquid investments with maturities of three months or less from date of purchase to be cash equivalents.



**Y-MABS THERAPEUTICS, INC.**

**Notes to Consolidated Financial Statements (Continued)**

**December 31, 2016**

**NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)**

The Company primarily maintains its cash balances with financial institutions in federally insured accounts and cash held in an unrestricted escrow account. The Company has cash in financial institutions in excess of FDIC insurance limits.

***Income Taxes***

The Company accounts for income taxes under the asset and liability approach for the financial accounting and reporting of income taxes. Deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to net operating loss carry forwards and temporary differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. These assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which the temporary differences are expected to reverse. A valuation allowance is established when management determines that it is more likely than not that some portion or all of the deferred tax assets will not be realized.

The Company prepares and files tax returns based on its interpretation of tax laws and regulations. In the normal course of business, the Company's tax returns are subject to examination by various taxing authorities. Such examinations may result in future tax and interest assessments by these taxing authorities. In determining the Company's tax provision for financial reporting purposes, the Company establishes a reserve for uncertain tax positions unless such positions are determined to be more likely than not of being sustained upon examination based on their technical merits. The Company considers many factors when evaluating and estimating its tax positions and tax benefits, which may require periodic adjustments and which may not accurately anticipate actual outcomes. Accordingly, the Company will report a liability for unrecognized tax benefits resulting from any uncertain tax positions taken or expected to be taken on a tax return.

The Company's policy is to recognize, when applicable, interest and penalties on uncertain tax positions as part of income tax expense.

***Research and Development Costs***

Research and development costs are charged to operations when incurred and are included in operating expenses. Research and development costs consist principally of compensation cost for our employees and consultants that perform our research activities, the fees paid to maintain our licenses, the payments to third parties for manufacturing and clinical research organizations and additional product development, and consumables and other materials used in research and development. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. Actual results could differ from the Company's estimates. The Company is obligated to make certain milestone and royalty payments in accordance with the contractual terms of the MSK License based upon the resolution of certain contingencies. The Company records the milestone and royalty payment when the achievement of the milestone or payment of the milestone or royalty is probable and the amount of the payment is reasonably estimable. Research and development costs were \$13,855,000 for the year ended December 31, 2016.

**Y-MABS THERAPEUTICS, INC.**

**Notes to Consolidated Financial Statements (Continued)**

**December 31, 2016**

**NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)**

***Patent Costs***

The Company expenses the costs of obtaining and maintaining patents as general and administrative expenses.

***Stock-Based Compensation***

The Company measures stock options granted to employees and directors based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company issues stock options with only service-based vesting conditions and records the expense for these awards using the straight-line method over the requisite service period.

For share-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of the Company's common shares and updated assumption inputs in the Black-Scholes option-pricing model.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information for its shares. Therefore, it estimates its expected share price volatility based on the historical volatility of a group of publicly-traded peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded share price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards as the Company has limited historical data to support the expected term assumption. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield is based on the fact that the Company has never paid cash dividends on common shares and does not expect to pay any cash dividends in the foreseeable future.

***Segment Information***

The Company is engaged solely in the discovery and development of novel antibody therapeutic products to treat cancer. Accordingly, the Company has determined that it operates in one operating segment

***Comprehensive Loss***

Comprehensive loss includes net loss as well as other changes in stockholders' equity that result from transactions and economic events other than those with shareholders. The difference between net loss and comprehensive loss for the period presented in the accompanying financial statements was due to foreign currency translation.

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)*****Foreign Currency Translation***

The financial statements of our Danish subsidiary with a functional currency other than the U.S. dollar are translated into U.S. dollars using period-end exchange rates for assets and liabilities, historical exchange rates for stockholders' equity and weighted average exchange rates for operating results. Translation gains and losses are included in accumulated other comprehensive income (loss), net of tax, in stockholders' equity. Foreign currency transaction gains and losses are included in the results of operations in other income and expense.

***Recently Issued Accounting Pronouncements***

In May 2017, the Financial Accounting Standards Board (the "FASB") issued Accounting Standards Update 2017-09 ("ASU 2017-09"), Compensation—Stock Compensation (Topic 718)—Scope of Modification Accounting. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award changes as a result of the change in terms or conditions. The guidance is effective prospectively for annual periods beginning on or after December 15, 2017 with early adoption permitted. The Company will account for any modifications in accordance with ASU 2017-09 subsequent to the effective date.

In November 2016, the FASB issued Accounting Standards Update 2016-18 ("ASU 2016-18"), Statement of Cash Flows (Topic 230)—Restricted Cash. Under the new guidance, it changes the presentation of restricted cash and cash equivalents on the statement of cash flows. Restricted cash and restricted cash equivalents will be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. This amendment is effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted, including adoption in an interim period. The Company is currently evaluating the impact of this new pronouncement on its statement of cash flows.

In August 2016, the FASB issued Accounting Standards Update 2016-15 ("ASU 2016-15"), Classification of Certain Cash Receipts and Cash Payments. ASU 2016-15 clarifies how certain cash receipts and payments should be presented in the statement of cash flows. The guidance is effective in 2018 with early adoption permitted. We are currently evaluating the timing of adoption of this guidance.

In March 2016, the FASB issued Accounting Standards Update No. 2016-09 ("ASU 2016-09"), Improvements to Employee Share-Based Payment Accounting, which changes the accounting for certain aspects of share-based payments to employees. The guidance requires the recognition in the income statement of the income tax effects of vested or settled awards. Further, the guidance requires that the recognition of anticipated tax windfalls/shortfalls be excluded in the calculation of assumed proceeds when applying the treasury stock method. The guidance also allows for the employer to repurchase more of an employee's shares for tax withholding purposes and not classify the award as a liability that requires valuation on a mark-to-market basis. In addition, the guidance allows for a policy election to account for forfeitures as they occur rather than on an estimated basis. The guidance is effective in 2017 with early adoption permitted and the Company elected to early adopt ASU 2016-09 effective January 1, 2016. Adoption of ASU 2016-09 did not have a material impact on its financial statements and related disclosures.

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)**

In February 2016, the FASB issued Accounting Standards Update No. 2016-02 ("ASU 2016-02"), Leases, which is effective for fiscal years, and interim periods within those years, beginning after December 15, 2018 with early adoption permitted. Under ASU 2016-02, lessees will be required to recognize for all leases, at the commencement date of the lease, a lease liability, which is a lessee's obligation to make lease payments arising from a lease measured on a discounted basis, and a right-to-use asset, which is an asset that represents the lessee's right to use or control the use of a specified asset for the lease term. The Company is currently evaluating the effect that the new guidance will have on its financial statements and related disclosures.

In November 2015, the FASB issued Accounting Standards Update 2015-17 ("ASU 2015-17"), Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes. ASU 2015-17 simplifies current guidance and requires companies to classify all deferred tax assets and liabilities as noncurrent on the balance sheet. ASU 2015-17 can be applied either prospectively or retrospectively and is effective for periods beginning after December 15, 2016, with early adoption permitted. The guidance will be effective for the Company's annual and interim reporting periods beginning January 1, 2017; however, the Company has concluded that the guidance will not have a material impact on the financial statements.

**NOTE 4—EARNINGS (LOSS) PER SHARE**

Basic earnings (loss) per share ("EPS") is calculated by dividing net income or loss attributable to common stockholders by the weighted average common stock outstanding. Diluted EPS is calculated by adjusting weighted average common shares outstanding for the dilutive effect of common stock options and warrants. In periods in which a net loss is recorded, no effect is given to potentially dilutive securities, since the effect would be antidilutive. Securities that could potentially dilute basic EPS in the future were not included in the computation of diluted EPS because to do so would have been antidilutive. The calculations of basic and diluted net loss per share are as follows:

	<u>2016</u> <u>(in thousands, except share and per share amounts)</u>
Net loss (numerator)	\$ (17,057)
Weighted-average shares, in thousands (denominator)	14,087,456
Basic and diluted net loss per share	\$ (1.21)

Potentially dilutive securities outstanding as of December 31, 2016 relate to stock options outstanding of 2,159,000.

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 5—ACCRUED LIABILITIES**

Accrued short-term liabilities at December 31, 2016 are as follows:

Accrued milestone payments	\$ 400,000
Accrued compensation and board fees	324,000
Other	24,000
Total	<u>\$ 748,000</u>

Accrued long-term liabilities at December 31, 2016 are as follows:

Accrued milestone and royalty payments	<u>\$ 2,225,000</u>
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**NOTE 6—LICENSE AGREEMENT AND COMMITMENTS**License Agreement

On August 20, 2015, we entered into the MSK License that grants us a worldwide, sublicensable license to MSK's rights to certain patent rights and intellectual property rights related to certain know-how to develop, make, and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments.

The patents and patent applications covered by this agreement are directed, in part, to naxitamab, an anti GD2 antibody, and omburtamab, which is an anti B3-H7 antibody, as well as affinity matured versions of certain antibodies and certain single chain variable fragments (Fv) constructs, and their use for immunotherapy, targeting the treatment of neuroblastoma and other pediatric oncology indications. Upon entering into the MSK License in 2015 and in exchange for the licenses, we paid MSK an upfront payment of \$500,000, issued 1,428,500 shares of our common stock to MSK and agreed to provide certain anti-dilution rights to MSK as further described in Note 7, Stockholders' Equity. In addition, we are required to pay to MSK certain royalty and milestone payments. We expensed the upfront payment and the issuance of shares to MSK in 2015. Total expense of \$285,700 was recorded for the shares of common stock issued to MSK in 2015 based on the estimated fair value of the shares of common stock of \$0.20 per share at issuance date. We also recorded expense related to common stock issued related to certain anti-dilution rights held by MSK. See further description in note 7.

The MSK License requires us to pay to MSK mid to high single digit royalties based on annual net sales of licensed products or the performance of licensed services by us and our affiliates and sublicensees. We are required to pay annual minimum royalties of \$80,000 over the royalty term, commencing on the fifth anniversary of the license agreement. These amounts are non-refundable but are creditable against royalty payments otherwise due under the MSK License. Total expensed minimum royalty payments in 2016 under the MSK License were \$1,200,000, all of which were recorded as long-term accrued liabilities as of December 31, 2016, upon determination that the payment of such minimum royalties was probable and the amount was estimable. We are also obligated to pay MSK certain clinical, regulatory and sales-based milestone payments under the MSK License. These milestone payments become due upon achievement of the related clinical, regulatory or sales-based milestone defined in the MSK License. Certain of the clinical and regulatory milestone payments

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 6—LICENSE AGREEMENT AND COMMITMENTS (Continued)**

become due at the earlier of completion of the related milestone activity or the date indicated in the MSK License. Total potential clinical and regulatory milestones potentially due under the MSK License are \$2,450,000 and \$9,000,000, respectively. There are also sales-based milestones that become due should YmAbs achieve certain amounts of sales of licensed products resulting from the license arrangement with MSK, with total sales-based milestones potentially due of \$20,000,000. The Company records milestones in the period in which the contingent liability is probable and the amount is reasonably estimable. In addition, to the extent we enter into sublicense arrangements, we are required to pay to MSK a percentage of certain payments that we receive from sublicensees of the rights licensed to us by MSK, which percentage will be based upon the date we receive such payments or the achievement of certain clinical milestones. The Company has not entered into any sublicenses related to the MSK License.

Failure by the Company to meet certain conditions under the arrangement could cause the related license to such licensed product to be canceled and could result in termination of the entire arrangement with MSK. One of such conditions required that the Company obtain financing in certain increments in 2015 and 2016, with a requirement that a total of \$25,000,000 of financing be raised by the Company on or before December 31, 2016, including certain limitations on the nature of such financing. Failure to meet the financing requirements under the arrangement would have caused the Company to be in breach of the MSK License and would be cause for its immediate termination. The Company obtained the financing as required under the agreement, including raising the cumulative total of \$25,000,000 in proceeds in 2016 through the issuance of shares of the Company's common stock which also triggered certain anti-dilution rights held by MSK. The common stock financing raised by the Company, the anti-dilution rights held by MSK and the associated expense recorded upon issuance of the shares related to the anti-dilution rights are further described in Note 7. In addition, the Company may terminate the MSK License with prior written notice to MSK. Total milestones expensed in 2016 under the arrangement with MSK were \$1,675,000, all of which related to clinical milestones, which become due either based upon the passage of time or achievement of the related milestone activities. Of these clinical milestones, \$250,000 was paid in 2016, and \$1,425,000 was recorded as accrued liabilities as of December 31, 2016. Of the \$1,425,000 recorded as accrued liabilities, \$1,025,000 was recorded within accrued long-term liabilities and \$400,000 was recorded within accrued short-term liabilities. These milestone-related charges were recorded as research and development expense. The Company determined that payment of these clinical milestone payments was probable in 2016, after satisfying the financing requirements described above. The Company also considered each party's termination rights under the agreement when considering whether any regulatory-based milestone payments, certain of which also contain time-based payment requirements, were probable.

On November 5, 2015, we entered into a sponsored research agreement, which we refer to as the SRA, with MSK pursuant to which we agreed to pay MSK to provide research services over a period of five years related to the intellectual property licensed under the MSK License. During 2016, we incurred research and development expenses of \$1,099,000 under the SRA.

On September 20, 2016, we entered into a master data services agreement, which we refer to as the MDSA, with MSK pursuant to which we committed to provide make certain payments in exchange for services provided by approximately two full time employees at MSK, who are engaged in

**Y-MABS THERAPEUTICS, INC.**

**Notes to Consolidated Financial Statements (Continued)**

**December 31, 2016**

**NOTE 6—LICENSE AGREEMENT AND COMMITMENTS (Continued)**

transferring clinical data, databases, regulatory files and other know-how included in the MSK License. During 2016, we incurred expenses of \$265,000 under the MDSA.

*Lease Agreements*

The Company maintains a six-month lease agreement in connection with its corporate headquarters in New York. The term of the lease is renewed every six months, with rent payable in monthly installments of approximately \$5,000, which are recognized on a straight-line basis. Additionally, the Company maintains a lease for certain office space in Denmark as further described in Note 9, Related Party Transactions.

**NOTE 7—STOCKHOLDERS' EQUITY**

***Authorized Stock***

As of December 31, 2016, the Company has authorized a total of 51,000,000 shares, 50,000,000 of which are to be common stock, par value \$0.0001 per stock, and 1,000,000 of which are to preferred stock, par value \$0.0001 per share.

***Common Stock***

Each share of common stock is entitled to one vote. Common stockholders are entitled to receive dividends, as may be declared by the board of directors, if any, subject to preferential dividend rights of the preferred stock, none of which have been issued. The Company has issued 16,552,884 shares of its common stock, par value \$0.0001 per share as of December 31, 2016.

***Preferred Stock***

Preferred stock may be issued from time to time in one or more series with such designations, preferences and relative participating, optional or other special rights and qualifications, limitations or restrictions as approved by the Company's Board of Directors. No preferred stock has been issued as of December 31, 2016.

***Issuances of common stock for MSK License***

In connection with the MSK License, in August 2015 we issued to MSK 1,428,500 shares of our common stock and the estimated fair market value of these shares was expensed when issued in 2015. We also agreed to provide certain anti-dilution rights to MSK. If at any time after such issuance, the Company issued any shares of its common stock, the Company was required to issue sufficient shares of common stock to MSK such that at all times prior to the Company obtaining equity financing equal to or greater than \$25,000,000 in the aggregate, MSK shall hold shares of the common stock of the Company equal to 12.5% of the issued and outstanding shares of common stock (assuming full conversion or exercise of all outstanding preferred stock and other convertible securities, rights, options and warrants).

In 2016, our aggregate equity financing reached \$25,000,000 since inception, and we issued to MSK a total of 999,929 shares of our common stock in order for MSK to retain 12.5% of the issued

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 7—STOCKHOLDERS' EQUITY (Continued)**

and outstanding shares of common stock until the \$25,000,000 has been raised. Shares were issued at the Company's estimated fair market value of such shares at the time of issuance, corresponding to \$4.38 per share, and the total value of \$4,380,000 was charged to expense in the period incurred when the anti-dilution rights were triggered, with \$2,280,000 recognized in 2016. Subsequent to the issuance of such shares and upon achievement of the financing requirement, there are no further anti-dilution rights due to MSK.

***Stock grant agreements with nonemployees***

In August 2015, we entered into certain stock grant agreements with non-employees of the Company. We agreed to issue a total of 2,800,000 shares to two non-employee doctors that were involved in the development of technology licensed from MSK in consideration for their prior service. These non-employees are employees of MSK. The shares are released according to a vesting schedule. A total of 560,000 shares were issued in 2015, with a total of 448,000 shares issued in 2016 and 448,000 each year thereafter until 2020, such that the total grant will have been issued. The total award was expensed at its estimated fair value of \$560,000 or \$0.20 per share in 2015 upon issuance, as no future service was required to continue to vest in and receive the shares of common stock. In August 2016, the Company subsequently repurchased and retired a total of 83,600 shares from the two nonemployees of the Company at an amount equal to \$4.38 per share. The transaction reduced the Company's shareholders' equity by \$366,000. The share vesting schedule for the stock grant agreements with such nonemployees shall be automatically accelerated upon a change in control or consummation of an initial public offering of the Company, as defined in the stock grant agreements.

***Issuance of common stock***

From March through July 2016, we issued 3,425,012 shares of Common Stock at a purchase price of \$4.38 per share for an aggregate consideration of \$14,961,000, net of issuance costs.

In December 2016, we issued 520,159 shares of common stock upon receipt of \$4,377,000, net of issuance costs, from investors at a purchase price of \$8.50 per share.

**NOTE 8—STOCK OPTIONS*****2015 Equity Incentive Plan***

Our board of directors and stockholders have approved and adopted our 2015 Equity Incentive Plan as amended and restated (2015 Equity Plan), which provides for the grant of incentive stock options, within the meaning of Section 422 of the Code (the Internal Revenue Code), to our employees and any parent and subsidiary corporations' employees, and for the grant of incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock and restricted stock units to our employees, directors and consultants and our parent and subsidiary corporations' employees and consultants. A total of 4,500,000 shares of our common stock are reserved for issuance pursuant to the 2015 Equity Plan. In addition, the number of shares available for issuance under the 2015 Equity Plan will also include an annual increase on the first day of each fiscal year beginning in 2016, equal to 6% of the outstanding shares of common stock as of the last day of our immediately preceding fiscal year.



## Y-MABS THERAPEUTICS, INC.

## Notes to Consolidated Financial Statements (Continued)

December 31, 2016

## NOTE 8—STOCK OPTIONS (Continued)

*Stock Options*

Stock options may be granted under the 2015 Equity Plan. The exercise price of options granted under the 2015 Equity Plan must at least be equal to the fair market value of our common stock on the date of grant. The term of an incentive stock option may not exceed 10 years, except that with respect to any participant who owns more than 10% of the voting power of all classes of our outstanding stock, the term must not exceed five years and the exercise price must equal at least 110% of the fair market value on the grant date. The administrator will determine the methods of payment of the exercise price of an option, which may include cash, shares or other property acceptable to the administrator, as well as other types of consideration permitted by applicable law. Options granted under the 2015 Equity Plan vest according to the schedule specified in the grant agreements, which is generally a four year period and generally become immediately exercisable upon the occurrence of a change in control, as defined.

During the year ended December 31, 2016, stock-based compensation expense for stock option grants was \$258,000 and was recorded as \$93,000 in research and development expense and \$165,000 in general and administrative expense.

*Stock Option Valuation*

The assumptions that the Company used to determine the fair value of the stock options granted to employees and directors were as follows, presented on a weighted average basis:

	Year Ended December 31, 2016
Risk-free interest rate	1.77%
Expected term (in years)	7.0
Expected volatility	60.6%
Expected dividend yield	0%

The Company recognizes compensation expense for only the portion of awards that are expected to vest.

The following table summarizes common stock options issued and outstanding:

	Options	Weighted average exercise price	Aggregate intrinsic value (in thousands)	Weighted average remaining contractual life (years)
Outstanding and expected to vest at December 31, 2015	1,300,000	\$ 2.00		
Granted	859,000	\$ 4.61		
Outstanding and expected to vest at December 31, 2016	<u>2,159,000</u>	<u>\$ 3.04</u>	<u>\$ 11,791</u>	<u>8.95</u>
Exercisable at December 31, 2016	<u>511,500</u>	<u>\$ 2.11</u>	<u>\$ 3,268</u>	<u>8.51</u>

The weighted average grant-date fair value of stock options granted during the year ended December 31, 2016 was \$2.78 per share.

**Y-MABS THERAPEUTICS, INC.****Notes to Consolidated Financial Statements (Continued)****December 31, 2016****NOTE 8—STOCK OPTIONS (Continued)**

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock.

As of December 31, 2016, we had \$2,176,000 of unrecognized compensation related to employee stock options that are expected to vest over a period of 2.88 years.

**NOTE 9—RELATED PARTY TRANSACTIONS**

During the year ended December 31, 2016, we paid a founding shareholder for expenses related to the Company of \$134,000.

MSK is a shareholder of the Company and under the MSK License, SRA and MDSA, we have expensed costs in the total amount of \$4,445,000 in the year ended December 31, 2016 for milestones, research costs and patent activities. Please refer to Note 6 for additional details on our license agreement with MSK. As of December 31, 2016, we had a total of \$3,050,000 recorded as accounts payable and accrued liabilities related to amounts due to MSK.

In July 2016, the Company entered into an agreement of lease with a shareholder of the Company, Weco Group, in connection with the subsidiary in Denmark. The lease payable thereunder is approximately \$4,000 per month and, as the lease can be terminated with three months' notice, any future rent commitment thereunder will amount to approximately \$12,000. In addition, the Company reimbursed Weco Group for certain administrative expenses. The total expenses, including rent, equaled \$44,000 for 2016.

**NOTE 10—INCOME TAXES**

Domestic and foreign loss before income taxes are as follows:

	<b>For The Year Ended December 31, 2016 (thousands)</b>
United States	\$ (16,915)
Foreign	(142)
Total	<u>\$ (17,057)</u>

The Company provided no current and deferred income taxes/(benefits) on net loss of (\$17,057,000) for year ended December 31, 2016.

## Y-MABS THERAPEUTICS, INC.

## Notes to Consolidated Financial Statements (Continued)

December 31, 2016

## NOTE 10—INCOME TAXES (Continued)

The difference between income taxes expected at the U.S. federal statutory income tax rate of 34% and income taxes provided are set forth below:

	December 31, 2016 (thousands)
Taxes on income at U.S. federal statutory rate	\$ (5,799)
State and local taxes, net of federal tax effects	(1,896)
Foreign tax rate differential	16
Valuation allowance	7,965
Tax credits	(286)
	<u>—</u>

Significant components of the Company's net deferred tax assets/(liabilities) are as follows:

	December 31, 2016 (thousands)
Deferred tax assets:	
Acquired Intangibles	\$ 2,984
Accrued royalty	542
Stock based compensation	116
Net operating loss carryforwards	6,450
Tax credit carryforwards	400
Total deferred tax assets	<u>10,492</u>
Valuation allowance	(10,492)
Net deferred tax assets	<u>—</u>

The Company recognizes income tax benefits for tax positions determined more likely than not to be sustained upon examination, based on the technical merits of the positions. As of December 31, 2016, the Company has determined that there were no uncertain tax positions. The Company's tax returns for years 2015 and 2016 are open for tax examination by U.S. federal and state, and the Danish tax authorities.

The valuation allowance related primarily to net U.S. deferred tax assets from operating losses, research and development tax credit carryforwards, and acquired intangibles.

The Company maintains a full valuation allowance on its U.S. and foreign deferred tax assets. The assessment regarding whether a valuation allowance is required considers both positive and negative evidence when determining whether it is more-likely-than-not that deferred tax assets are recoverable. In making this assessment, significant weight is given to evidence that can be objectively verified. In its evaluation, the Company considered its cumulative loss in recent years and its forecasted losses in the near-term as significant negative evidence. Based upon review of available positive and negative evidence, the Company determined that the negative evidence outweighed the positive

**Y-MABS THERAPEUTICS, INC.**

**Notes to Consolidated Financial Statements (Continued)**

**December 31, 2016**

**NOTE 10—INCOME TAXES (Continued)**

evidence and a full valuation allowance on its U.S. and foreign deferred tax assets will be maintained. The Company will continue to assess the realizability of its deferred tax assets and will adjust the valuation allowance as needed.

As of December 31, 2016, the Company had U.S. federal and state and local net operating loss ("NOL") carryforwards of approximately \$14,192,000, which are available to reduce future taxable income. The Company also had U.S. federal tax credits of \$400,000 as of December 31, 2016, which may be used to offset future tax liabilities. The NOL and tax credit carryforwards will begin to expire in 2035. The NOL and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986 ("IRC"). This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. Subsequent ownership changes may further affect the limitation in future years. The Company also has Danish NOL carryforwards of \$150,000, which have an indefinite carryforward period.

**NOTE 11—OTHER BENEFITS**

The Company has established a retirement program for the employees of our Danish subsidiary pursuant to which all such employees can contribute an amount at their election from their base compensation and may receive contributions from our Danish subsidiary. Contributions from our Danish subsidiary were immaterial during the year ended December 31, 2016. In addition, health insurance benefits for our Danish employees are fully paid for by such employees. Our Danish subsidiary does not incur any costs for these health insurance benefits.

**NOTE 12—SUBSEQUENT EVENTS**

The Company has evaluated subsequent events through February 13, 2018, the date that these consolidated financial statements were issued.

- [1] In January and February 2017, the Company issued 1,192,662 shares of common stock at \$8.50 per share to investors for proceeds of \$10,138,000.
- [2] In June 2017, the Company entered into an Investigator-Sponsored Master Clinical Trial Agreement, which we refer to as the MCTA, with MSK pursuant to which we committed to pay for research services, in connection with clinical studies and related activities for the antibodies we licensed under the license arrangement with MSK over a five year period. Amounts payable to MSK under the MCTA are subject to agreement between the Company and MSK on budgets for individual studies.
- [3] In July 2017, the Company's Board of Directors increased the number of authorized shares available to be issued by the Company to a total of 55,500,000 shares, of which 50,000,000 shares are to be shares of common stock and 5,500,000 are to be shares of preferred stock.
- [4] In October 2017 the Company issued 5,347,568 shares of common stock at \$9.35 per share to investors for proceeds of \$50,000,000.

**Y-MABS THERAPEUTICS, INC.**

**Notes to Consolidated Financial Statements (Continued)**

**December 31, 2016**

**NOTE 12—SUBSEQUENT EVENTS (Continued)**

- [5] In November 2017 the Company issued 3,208,556 shares of common stock at \$9.35 per share to investors for proceeds of \$30,000,000.
- [6] In November 2017, the Company entered in an exclusive license agreement with MSK related to certain CD33 antibodies and bispecific constructs thereof developed in the laboratory of a specific principal investigator at MSK for use in potential anti-cancer treatments. The license agreement contains certain conditions under which MSK or the Company may terminate the agreement. The agreement contains requirements for the Company to make certain milestone payments to MSK upon the achievement of related milestone clinical, regulatory approval and sales based activities. Certain of the milestone payments become due at the earlier of completion of the related milestone activity or the date indicated in the license agreement. Total potential milestones due under the arrangement are \$550,000, \$500,000 and \$7,500,000 for clinical, regulatory and sales based milestones, respectively. In addition, the arrangement contains minimum royalty payments that become due beginning in year 10 of \$40,000 per year prior, subject to increase and creditable against any royalty payments due based on sales in the future. The Company is required to pay mid to high single digit royalties on sales of licensed products. The Company also agreed to pay MSK approximately \$1,360,000 for research services related to the intellectual property licensed by the Company in this agreement. The research services are expected to occur over the two year period immediately following the entering into this arrangement.
- [7] On December 22, 2017, H.R.1 "An Act to Provide for Reconciliation Pursuant to Titles II and V of the Concurrent Resolution on the Budget for Fiscal Year 2018" ("tax reform") was signed by the President of the United States and became enacted law. Tax reform is complex and includes various changes which could impact the Company. For example, the 2017 Act reduces the corporate tax rate from 35% to 21% for tax years beginning after December 31, 2017 and as such deferred taxes will need to be remeasured based upon the 21% tax rate. The Company will also need to adjust valuation allowance accordingly. For net operating losses (NOLs) arising after December 31, 2017, the 2017 Act limits a taxpayer's ability to utilize NOL carryforwards to 80% of taxable income. In addition, NOLs arising after 2017 can be carried forward indefinitely, but carryback is generally prohibited. NOLs generated in tax years beginning before January 1, 2018 will not be subject to the taxable income limitation and will continue to have a two year carryback and 20 year carryforward period. The Company will continue to evaluate the impact of tax reform and record its measurement of income tax effects in the 2017 and 2018 financial statements.
- [8] In January 2018, the Company entered into a new lease agreement in connection with its corporate headquarters in New York. The term of the lease is five years from date the Company begins to occupy the premises. Fixed rent payable under the lease is approximately \$384,000 per annum and is payable in equal monthly installments of approximately \$32,000.

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Through and including \_\_\_\_\_, 2018 (the 25th day after the date of this prospectus), all dealers effecting transactions in the common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

**Shares**



**Common Stock**

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**PROSPECTUS**

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**BofA Merrill Lynch**

**Cowen**

, 2018

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**PART II****INFORMATION NOT REQUIRED IN PROSPECTUS****Item 13. Other Expenses of Issuance and Distribution.**

The following table sets forth the expenses to be incurred in connection with the offering described in this registration statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimates except the Securities and Exchange Commission's registration fee, the Financial Industry Regulatory Authority, Inc. filing fee and the Nasdaq listing fee.

	<u>Amount</u>
Securities and Exchange Commission registration fee	\$ *
Financial Industry Regulatory Authority, Inc. filing fee	*
Nasdaq listing fee	*
Accountants' fees and expenses	*
Legal fees and expenses	*
Transfer Agent's fees and expenses	*
Printing and engraving expenses	*
Miscellaneous fees and expenses	*
<b>Total expenses</b>	<b>\$ *</b>

\* To be filed by amendment.

**Item 14. Indemnification of Directors and Officers.**

Section 102 of the DGCL permits a corporation to eliminate the personal liability of its directors for monetary damages for a breach of fiduciary duty as a director, except where the director breached his or her duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of the DGCL or obtained an improper personal benefit. Upon completion of this offering, our certificate of incorporation will provide that none of our directors shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability, except to the extent that the DGCL prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the DGCL provides that a corporation has the power to indemnify a director, officer, employee, or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he or she is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses that the Court of Chancery or such other court shall deem proper.

Upon the completion of this offering, our certificate of incorporation will provide that we will indemnify each person who was or is a party or is threatened to be made a party or is involved in any

threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of us) by reason of the fact that he or she is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (all such persons being referred to as an "Indemnitee"), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding and any appeal therefrom, if such Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, and, with respect to any criminal action or proceeding, he or she had no reasonable cause to believe his or her conduct was unlawful. Our certificate of incorporation that will be effective as of the closing date of this offering also provides that we will indemnify any Indemnitee who was or is a party to an action or suit by or in the right of us to procure a judgment in our favor by reason of the fact that the Indemnitee is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise, or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding, and any appeal therefrom, if the Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to us, unless a court determines that, despite such adjudication but in view of all of the circumstances, he or she is entitled to indemnification of such expenses. Notwithstanding the foregoing, to the extent that any Indemnitee has been successful, on the merits or otherwise, he or she will be indemnified by us against all expenses (including attorneys' fees) actually and reasonably incurred by him or her or on his or her behalf in connection therewith. If we do not assume the defense, expenses must be advanced to an Indemnitee under certain circumstances.

We plan to enter into indemnification agreements with each of our executive officers and directors. In general, these agreements provide that we will indemnify the director or executive officer to the fullest extent permitted by law for claims arising in his or her capacity as a director or executive officer of our company or in connection with their service at our request for another corporation or entity. The indemnification agreements also provide for procedures that will apply in the event that a director or executive officer makes a claim for indemnification and establish certain presumptions that are favorable to the director or executive officer.

We maintain a general liability insurance policy that covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers.

The underwriting agreement we will enter into in connection with the offering of common stock being registered hereby provides that the underwriters will indemnify, under certain conditions, our directors and officers (as well as certain other persons) against certain liabilities arising in connection with such offering.

Insofar as the foregoing provisions permit indemnification of directors, executive officers, or persons controlling us for liability arising under the Securities Act of 1933, as amended, or the Securities Act, we have been informed that, in the opinion of the Securities and Exchange Commission, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.



**Item 15. Recent Sales of Unregistered Securities.**

Set forth below is information regarding shares of our common stock and shares of our preferred stock issued, and stock options granted, by us within the past three years that were not registered under the Securities Act. Included is the consideration, if any, we received for such shares and options and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

**(a) Stock Grants**

In June 2015, we issued and granted 2,190,000 shares of our common stock pursuant to various stock grant agreements.

In August 2015, we issued and granted 4,144,900 shares of our common stock pursuant to various stock grant agreements.

In May 2016, we issued and granted 479,328 shares of our common stock pursuant to various stock grant agreements.

In August 2016, we issued and granted 520,601 shares of our common stock pursuant to various stock grant agreements.

**(b) Issuance and Sale of Shares of Common Stock**

In August 2015, we issued and sold 5,010,000 shares of our common stock at a purchase price of \$0.20 per share for an aggregate purchase price of approximately \$1,002,000.

In November 2015, we issued and sold 1,027,397 shares of our common stock at a purchase price of \$4.38 per share for an aggregated purchase price of approximately \$4,499,999.

In December 2015, we issued and sold 1,027,487 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$4,500,400.

In March 2016, we issued and sold 570,776 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$2,500,000.

In April 2016, we issued and sold 1,261,412 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$5,525,000.

In May 2016, we issued and sold 515,204 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$2,256,600.

In June 2016, we issued and sold 890,406 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$3,900,000.

In July 2016, we issued and sold 187,214 shares of our common stock at a purchase price of \$4.38 per share for an aggregate purchase price of approximately \$820,000.

In December 2016, we issued and sold 578,982 shares of our common stock at a purchase price of \$8.50 per share for an aggregate purchase price of approximately \$4,921,396.

In January 2017, we issued and sold 1,016,486 shares of our common stock at a purchase price of \$8.50 per share for an aggregate purchase price of approximately \$8,640,172.

In February 2017, we issued and sold 117,353 shares of our common stock at a purchase price of \$8.50 per share for an aggregate purchase price of approximately \$997,500.

In October 2017, we issued and sold 5,347,568 shares of our common stock at a purchase price of \$9.35 per share for an aggregate purchase price of approximately \$49,999,923.

In November 2017, we issued and sold 3,208,552 shares of our common stock at a purchase price of \$9.35 per share for an aggregate purchase price of approximately \$30,000,000.

No underwriters were involved in the foregoing issuances of securities. The securities described in sections (a) and (b) of this Item 15 were issued in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the economic and other risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

**(c) Stock Option Grants and Option Exercises**

Between June 6, 2015 and December 14, 2016, we granted options to purchase an aggregate of 2,159,000 shares of common stock, with exercise prices ranging from \$2.00 to \$8.50 per share, to employees, directors, consultants and advisors pursuant to our 2015 Plan, as amended. As of the date hereof, no options have been exercised.

No underwriters were involved in the foregoing issuances of securities. The issuances of stock options and the shares of our common stock issued upon the exercise of the options described in section (c) of this Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, directors, consultants and advisors, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(a)(2) under the Securities Act relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

All of the securities described in sections (a), (b) and (c) of this Item 15 are deemed restricted securities for purposes of the Securities Act. All of the certificates representing such securities included appropriate legends setting forth that the securities have not been registered and the applicable restrictions on transfer.

**Item 16. Exhibits and Financial Statement Schedules.**

**(a) Exhibits**

The exhibits to the registration statement are listed in the Exhibit Index attached hereto and incorporated by reference herein.

**(b) Financial Statement Schedules**

No financial statement schedules are provided because the information called for is not required or is shown either in the financial statements or the related notes.

**Item 17. Undertakings.**

- (a) The undersigned registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

- (b) Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.
- (c) The undersigned registrant hereby undertakes that:
- (1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.
  - (2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

**EXHIBIT INDEX**

<b>Exhibit Number</b>	<b>Description of Exhibit</b>
1.1*	Form of Underwriting Agreement
3.1*	Certificate of Incorporation of the Registrant, as amended
3.2*	Bylaws of the Registrant
3.3*	Form of Amended and Restated Certificate of Incorporation of the Registrant (to be effective upon the closing of this offering)
3.4*	Form of Amended and Restated Bylaws of the Registrant (to be effective upon the closing of this offering)
4.1*	Specimen stock certificate evidencing the shares of common stock
4.2*	Registration Rights Agreement, dated as of October 13, 2017, among the Registrant and the other parties thereto
4.3*	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto
5.1*	Opinion of Satterlee Stephens LLP
10.1+*	License Agreement, dated as of August 20, 2015, by and between the Registrant and Memorial Sloan Kettering Cancer Center
10.2+*	License Agreement, dated as of November 13, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center
10.3†*	Amended and Restated 2015 Equity Incentive Plan
10.4†*	Form of Incentive Stock Option Agreement under the Amended and Restated 2015 Equity Incentive Plan
10.5†*	Form of Nonstatutory Stock Option Agreement under the 2015 Equity Incentive Plan
10.6†*	2017 Employee Stock Purchase Plan
10.7†*	Form of Director and Officer Indemnification Agreement
10.8†*	Service Agreement, effective as of April 1, 2016 between the Registrant and Thomas Gad
10.9†*	Service Agreement, effective as of March 1, 2016 between the Registrant and Dr. Claus Juan Møller San Pedro, M.D., Ph.D.
10.10†*	Service Agreement, effective as of October 1, 2015 between the Registrant and Bo Kruse
10.11*	Lease Agreement, dated January 10, 2018, by and between the Registrant and RXR HB Owner LLC
10.12*	Lease Agreement, effective as of July 1, 2016, by and between Y-mabs Therapeutics, A/S and Weco Management ApS
16.1*	Letter of PricewaterhouseCoopers Statsautoriseret Revisionspartnerselskab, independent registered public accounting firm.
21.1*	Subsidiaries of the Registrant
23.1*	Consent of PricewaterhouseCoopers LLP, independent registered public accounting firm

<u>Exhibit Number</u>	<u>Description of Exhibit</u>
23.2*	Consent of Satterlee Stephens LLP (included in Exhibit 5.1)
24.1*	Power of Attorney (included on signature page)

\* To be filed by amendment.

+ Confidential treatment requested as to certain portions, which portions have been omitted and filed separately with the Securities and Exchange Commission.

† Indicates a management contract or compensation plan.

**SIGNATURES**

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of New York, State of New York, on this \_\_\_\_\_ day of \_\_\_\_\_, 2018.

Y-MABS THERAPEUTICS, INC.

By:

\_\_\_\_\_  
Thomas Gad  
*Founder, Chairman, President and Head of Business  
Development*

**Power of Attorney**

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Thomas Gad and Claus Juan Møller San Pedro and each of them, his true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution for him and in his name, place and stead, in any and all capacities, to sign any and all amendments (including post-effective amendments) to this registration statement, and any subsequent registration statements pursuant to Rule 462 of the Securities Act and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that each of said attorney-in-fact or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

<u>Signature</u>	<u>Title</u>	<u>Date</u>
_____ Thomas Gad	Founder, Chairman of the Board of Directors, President and Head of Business Development	
_____ Claus Juan Møller San Pedro, M.D., Ph.D.	Chief Executive Officer, (principal executive officer) and Director	
_____ Bo Kruse	Executive Vice President, Chief Financial Officer, Secretary Treasurer (principal financial and principal accounting officer) and Director	

<u>Signature</u>	<u>Title</u>	<u>Date</u>
_____ Johan Wedell-Wedellsborg	Director	
_____ Gregory Raskin, M.D.	Director	
_____ Michael Buschle, Ph.D.	Director	
_____ James Healey, M.D.	Director	
_____ Ashutosh Tyagi, M.D.	Director	
_____ David N. Gill	Director	

