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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2018.

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to

Commission file number 001-38650

Y-mAbs Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**47-4619612**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)
230 Park Avenue, Suite 3350 New York, NY	**10169**
(Address of Principal Executive Offices)	(Zip Code)

Registrant's telephone number, including area code **(646)-885-8505**

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of each exchange on which registered**
Common Stock $0.0001 par value	NASDAQ Global Select Market

Securities registered pursuant to Section 12(g) of the Act: None

NONE

(Title of class)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.
Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act.
Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days.
Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files).
Yes ☒ No ☐

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§299.405 of this chapter) is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☒

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.

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒ 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 pursuant to Section 13(a) of the Exchange Act. ☒

Indicate by check mark whether the registrant is a shell company (as defined by Rule 12b-2 of the Exchange Act).
Yes ☐ No ☒

The aggregate market value of the common stock held by non-affiliates of the registrant, based on the closing price of a share of common stock on September 21, 2018 as reported by the NASDAQ Global Select Market on such date was approximately $301.6 million. The registrant has elected to use September 21, 2018, which was the initial trading date on the NASDAQ Global Select Market, as the calculation date because on June 30, 2018 (the last business day of the registrant's most recently completed second fiscal quarter), the registrant was a privately held company. Shares of the registrant's common stock held by each executive officer, director and holder of 5% or more of the outstanding common stock have been excluded in that such persons may be deemed to be affiliates. This calculation does not reflect a determination that certain persons are affiliates of the registrant for any other purpose. The number of outstanding shares of the registrant's common stock as of March 14, 2019 was 34,193,666.

Documents Incorporated by Reference:

Portions of the Registrant's Definitive Proxy Statement relating to the 2019 Annual Meeting of Stockholders, which will be filed with the Securities and Exchange Commission within 120 days after the end of the Registrant's fiscal year ended December 31, 2018 are incorporated by reference into Part III of this Report.

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FORWARD LOOKING STATEMENTS.

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, including statements regarding our business strategy, future operations and results thereof, future financial position, future revenue, projected costs, prospects, current and prospective products, product approvals, research and development costs, current and prospective collaborations, timing and likelihood of success, plans and objectives of management, expected market growth and future results of current and anticipated products, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “contemplate,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “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 two lead product candidates and other product candidates, including the potential clinical efficacy and other benefits thereof;
- our ongoing and future clinical trials for our two lead product candidates and other product candidates, whether conducted by us or by any of our collaborators, including the timing of initiation of these trials, the pace of enrollment, the completion of enrollment, the availability of data from these trials, the expected dates of BLA submission and approval by FDA and equivalent foreign regulatory authorities;
- 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, the pace of enrollment, the expected date of completion and of the anticipated results;
- the timing of and our ability to obtain and maintain regulatory, marketing and reimbursement 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 levels 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, including a direct salesforce in the future;
- 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 our cash and cash equivalents, how long that cash is expected to last, and the need for, timing and amount of any future financing transaction;
- our financial performance, including our estimates regarding revenues, expenses, capital expenditure requirements;
- 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 Annual Report on Form 10-K, 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.

The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we undertake no obligation to publicly update or review any forward-looking statement, whether as a result of new information, future developments or otherwise.

PART I

ITEM 1. 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 BLA for each of our two lead product candidates in 2019, with a goal of receiving approval by the FDA in 2020. 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 huB7-H3, a humanized version of omburtamab, each targeting indications with large adult patient populations. In addition, we have initiated a Phase I trial with our huGD2 BsAb product candidate for the treatment of refractory GD2 positive adult and pediatric solid tumours, thereby addressing large patient populations. We are also advancing a pipeline of novel BsAbs through late pre-clinical development, including our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. We believe that our BsAbs have the potential to result in improved tumor-binding, longer serum half-life 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, subtype 1□□□or IgG1κ, monoclonal antibody or mAb 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 multicenter 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, a pilot study (Study 17-251) of chemoimmunotherapy for high-risk 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 the dose escalation part of 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 duration of response of 12-weeks or longer. We have proposed to the FDA that, pending comparability between the study population 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. In May 2018, we reported topline results from the Phase 2 part of Study 12-230. The endpoints of this part of the study were complete tumor response (also known as complete remission), or CR, or partial tumor response, or PR. Complete tumor response is the total disappearance of a tumor and partial tumor response is a decrease in the size of a tumor, or in the extent of cancer in the body, in response to the treatment. This data continued to show response rates at the same levels as in the dose escalation part of the study with 13 of 15 evaluable, or 87% of, primary refractory patients responding and 7 of 23 evaluable, or 30% of, secondary refractory patients responding. We expect to submit the BLA for naxitamab for R/R high-risk NB in 2019. 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. In addition, on August 20, 2018, naxitamab received breakthrough therapy designation, or BTD, in combination with GM-CSF, for the treatment of high-risk NB refractory to initial therapy or with incomplete response to salvage therapy in patients greater than 12 months of age with persistent, refractory disease limited to bone marrow with or without evidence of concurrent bone involvement. Finally, in November 2018, the European Commission granted orphan medicinal product designation, or ("OMPD") for naxitamab 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. ^{131}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 who have CNS/LM from NB. As of August 2017, 93 patients with pediatric CNS/LM from NB had been treated with ^{131}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 in Study 03-133 and Study 101, data from both studies may be pooled for analysis for our planned BLA submission. ^{131}I-omburtamab has received ODD and RPDD from the FDA for the treatment of NB, and BTD for the treatment of pediatric patients who have CNS/LM from NB. In 2019, we expect to submit the BLA for ^{131}I-omburtamab for CNS/LM from NB.

^{124}I-omburtamab, which is omburtamab radiolabeled with Iodine-124, is currently being studied for the treatment of Diffuse Intrinsic Pontine Glioma, or DIPG. ^{131}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 and the EU 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 initiated Study 101 and Study 201 to form the primary basis for our planned BLAs, to establish comparability of study population and pharmacokinetics analysis with Study 03-133 and Study 12-230, respectively, and

to satisfy the confirmatory study and post-marketing requirements by the FDA. If the results from Study 101 and Study 201 fail to demonstrate comparability to the satisfaction of the FDA and other comparable regulatory authorities, this may lead to a delay in, or otherwise adversely affect, such clinical trials, including the timing of submission of BLAs.

We have two additional product candidates targeting B7-H3 in pre-clinical development, omburtamab-DTPA (diethylenetriamine pentaacetate), a Lutetium-177 conjugated antibody, and huB7-H3, a humanized version of omburtamab, each targeting indications with pediatric and 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 huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. As of December 10, 2018, FDA cleared the Investigational New Drug ("IND") application for the humanized bispecific GD2 antibody, and a Phase I trial was initiated with our huGD2-BsAb product candidate for the treatment of refractory GD2-positive adult and pediatric solid tumors. In pre-clinical studies, huGD2-BsAb has demonstrated the potential for improved tumor-binding, longer serum half-life and significantly greater T-cell mediated killing compared to existing bispecific constructs.

We currently have four active INDs related to our product candidates. The table below sets forth the product candidate, date of the initial submission of the IND to the FDA, as well as the current sponsor, the subject matter and the current status of each such IND.

Product Candidate	Date of Initial Submission	Current Sponsor	Subject Matter of IND	Current Status
Naxitamab	June 14, 2011	MSK	Treatment of NB and other GD2 positive tumors	Clinical trials ongoing
Omburtamab (^{131}I-Omburtamab and ^{124}I-Omburtamab)	September 22, 2000	Y-mAbs (MSK original sponsor)	CNS/LM from NB, DSRCT, DIPG and other B7-H3 positive tumors	Clinical trials ongoing
Naxitamab	September 5, 2017	Y-mAbs	Pediatric NB	Clinical trials ongoing
huGD2-BsAb	April 20, 2018	MSK	GD2 positive solid tumors	Clinical trial ongoing

In October 2017, the FDA issued a partial clinical hold on our IND for naxitamab. A partial clinical hold, as opposed to a full clinical hold, is a delay or suspension of only a specific part of the clinical work requested under the IND, which allows otherwise unaffected parts of the clinical work to proceed under the IND. The FDA stated that the proposed acceptance criterion for the ADCC-CD16, ADCC-CD32, and CDC assays were too wide to provide sufficient control over these attributes, which are critical for safety and efficacy. ADCC and CDC refer to antibody dependent cell-mediated cytotoxicity and complement-dependent cytotoxicity, respectively. We submitted a response to the FDA in March 2018, and met with the FDA in April, 2018. Subsequently, we submitted a complete response to the partial clinical hold to the FDA in May 2018 and the partial clinical hold was removed in June 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. 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. In addition, we believe that our relationship with MSK may help us 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. In 2006, Mr. Gad's then two year old daughter was diagnosed with high-risk NB and was treated at MSK with the murine version of naxitamab. In 2009, she relapsed with CNS/LM from NB and again was treated at MSK, this time with ^{131}I-omburtamab. Since then, she has been disease free. Our Chief Executive Officer, Dr. Claus Juan Møller San Pedro, was the co-founder of Genmab A/S, one of the largest public biotechnology companies in Europe, where he served as Executive Vice President and Chief Operating Officer for approximately 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 and Head 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 Senior Vice President and Chief Medical Officer, Dr. Steen Lisby, also comes from Genmab where he was Vice President, Medical Lead 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 $230.0 million from our initial public offering and 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 Investments, Inc., among others, and as of December 31, 2018, we had cash and cash equivalents of $147.8 million.

Our Pipeline

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

Product Candidate	Target	Study	Indication / Treatment	Preclinical	Phase 1	Phase 2	Phase 3 / Registration	Next Anticipated Milestone(s)
Naxitamab	GD2	201	Relapsed / Refractory High-Risk Neuroblastoma (Pediatric)			*Ongoing pivotal Phase 2 trial*[1]		2019-BLA Submission
		12-230	Relapsed / Refractory High-Risk Neuroblastoma (Pediatric)			*Ongoing Phase 2 trial*		
		16-1643	(Front-Line) High-Risk Neuroblastoma (Pediatric)			*Ongoing Phase 2 trial*		
		15-096	Relapsed (Second-Line) Osteosarcoma[2]			*Ongoing Phase 2 trial*		
		17-251	Chemoimmunotherapy for Relapsed / Refractory High-Risk Neuroblastoma			*Ongoing Phase 1 trial*		
Omburtamab	B7-H3	101	CNS / Leptomeningeal Metastases from Neuroblastoma (Pediatric) (^{131}I)[3]			*Ongoing pivotal Phase 2 trial*[4]		2019-BLA Submission
		03-133	Intrathecal Immunotherapy for CNS / Leptomeningeal Metastases (^{131}I)[3]			*Ongoing Phase 1 trial*		
		11-011	Diffuse Intrinsic Pontine Glioma (Pediatric) (^{124}I)[3]			*Ongoing Phase 1 trial*		
		09-090	Desmoplastic Small Round Cell Tumor (Pediatric) (^{131}I)[3]			*Ongoing Phase 1 trial*		
Omburtamab-DTPA[5]	B7-H3		B7-H3 Positive CNS / Leptomeningeal Solid Tumors					
huB7-H3	B7-H3		Systemic Solid Tumors (Adult) (Third-Line)					
huGD2-BsAb	GD2xCD3		Refractory GD2-Positive Solid Tumors			*Ongoing Phase 1 trial*		
huCD33-BsAb	CD33xCD3		Hematological Cancers Expressing CD33					

1. Pivotal registration study designed to support a BLA submission to the FDA, comprised of Study 12-230 measuring pharmacokinetic, toxicity and efficacy and an additional pivotal multicenter Phase 2 study, Study 201, designed to prove comparability between study sites using a current good manufacturing practices, or cGMP, commercial manufacturer. Study 201 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 designed to support a BLA submission to the FDA, comprised of Study 03-133 measuring pharmacokinetic, toxicity and efficacy and an additional pivotal multicenter Phase 2 study, Study 101, designed to prove comparability between study sites using a cGMP commercial manufacturer. Study 101 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.

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 ^{131}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 under their respective Break Through Designations, or BTD. We expect to submit a BLA for each of our two lead product candidates in 2019, with a goal of receiving approval by the FDA in 2020. 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 ^{131}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 to enter the clinic, huGD2-BsAb, is a bivalent humanized anti-GD2 and anti-CD3 BsAb. We are also advancing our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33 and expect to file an IND in 2020. 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.

- **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, supressor 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 2019, with a goal of receiving approval by the FDA in 2020. 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 B7-H3 targeting product candidates in pre-clinical development, omburtamab-DTPA and huB7-H3, a humanized version of omburtamab, each targeting indications with large adult cancer patient populations where there is a significant unmet medical need. As of December 10, 2018, FDA cleared the Investigational New Drug (“IND”) application for the humanized bispecific GD2 antibody or huGD2-BsAb, and we have initiated a Phase I trial with our huGD2-BsAb product candidate for the treatment of refractory GD2-positive adult and pediatric solid tumors. In pre-clinical studies, huGD2-BsAb has demonstrated the potential for improved tumor-binding, longer serum half-life and significantly greater T-cell mediated killing compared to existing bispecific constructs. We are also advancing a pipeline of novel BsAbs through late pre-clinical development, including our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. 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 was granted BTD in this indication in 2018. In November 2018, the European Commission granted orphan medicinal product designation, or OMPD, for naxitamab for the treatment of NB. Naxitamab has also 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. In the Phase 1 dose escalation part of 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 May 2018, we reported topline results from the Phase 2 part of Study 12-230. This data continued to show response rates at the same levels as in the dose escalation part of the study with 13 of 15 evaluable, or 87% of, primary refractory patients responding and 7 of 23 evaluable, or 30% of, secondary refractory patients responding.

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, the 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 duration of response of minimum 12 weeks may qualify naxitamab for accelerated approval. We have proposed to the FDA that, pending comparability between the study population 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, a pilot study (Study 17-251) of chemoimmunotherapy for high-risk 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 was granted BTD in this indication in 2018. In November 2018, the European Commission granted orphan medicinal product designation, or OMPD, for naxitamab for the treatment of NB. Naxitamab has also 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 the dose escalation part of 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 duration of response of minimum 12 weeks may qualify for consideration of an expedited approval of naxitamab. We have proposed to the FDA that, pending comparability between the study population 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 higher doses administered on an outpatient basis.

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.

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 six 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 high-risk 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 high-risk NB in Europe each year. We believe the current addressable market for naxitamab consists of approximately 960 new front-line high-risk NB patients each year and 675 primary or second-line eligible R/R NB pediatric 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 typically patients will receive five to 10 treatment cycles of naxitamab, each cycle consisting of 3 doses.

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. In 2017 the EMA approved Dinutuximab beta Apeiron (also known as dinutuximab beta, ch14.18/CHO, Isqette and currently being commercialized under the name Qarziba® in Europe), 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 the last 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 2019.

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 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 duration of response of minimum 12 weeks 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 1/2 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.

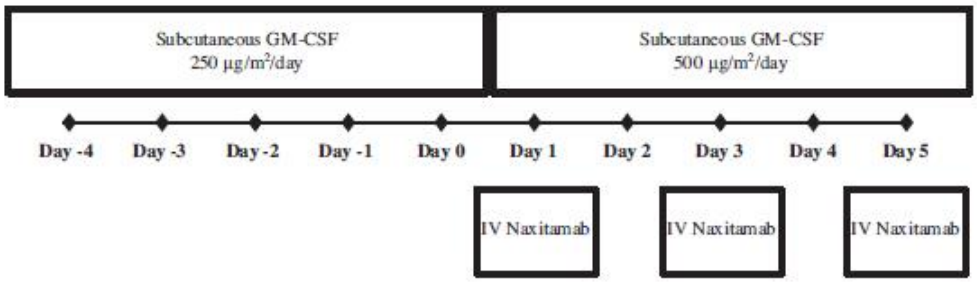
Patients must have R/R high-risk NB (including MYCN-amplified Stage 2, 3, 4, or 4S of any age and MYCN-non amplified Stage 4 in patients over 18 months of age) resistant to standard therapy. Standard therapy for these types of patients includes intensive induction chemotherapy, followed by a variety 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 who completed 4 cycles without PD were eligible to continue treatment for up to 24 months. For the Phase II part of study, patient were eligible to continue treatment for up

to 4 cycles after major clinical response was obtained again with a maximum treatment period of 24 months. 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%)
^{131}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 ^{131}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. Dose limiting toxicities, or DLTs, occurred in four of 57 patients. These DLTs did not show any consistent pattern, ranging from elevated liver enzymes, anaphylactic reactions, acute renal failure, and hypertension. Thirty-three patients experienced a total of 150 SAEs, of which 27 SAEs were treatment-related, and none were fatal. Two patients experienced Grade 4 toxicity that necessitated withdrawal from the study. One patient developed an anaphylactic reaction at cycle 7. Another one patient developed Grade 4 angioedema immediately after completing the second cycle. All 57 patients experienced at least one Treatment Emergent Adverse Event, or TEAE, which is defined as “an event that emerges during treatment, having been absent pretreatment, or worsens relative to the pretreatment state” of any grade. Most frequently observed TEAEs were pain, hypotension, fever, pruritus, and urticaria. Most TEAEs were low-grade adverse events.

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, 10 patients developed human anti-human antibody, or HAHA, response. Of the same 57 patients, 47 patients had previously been exposed to anti-GD2 based therapies, including the earlier murine version of naxitamab.

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 partial response or PR, which resulted in an ORR of 57% (13/23). Further, one patient had stable disease (cancer that is neither decreasing nor increasing in extent or severity) or SD, another six patients had progressive disease (cancer that is growing, spreading or getting worse) or PD, and two patients were only available for short term follow-up (long term data not available).

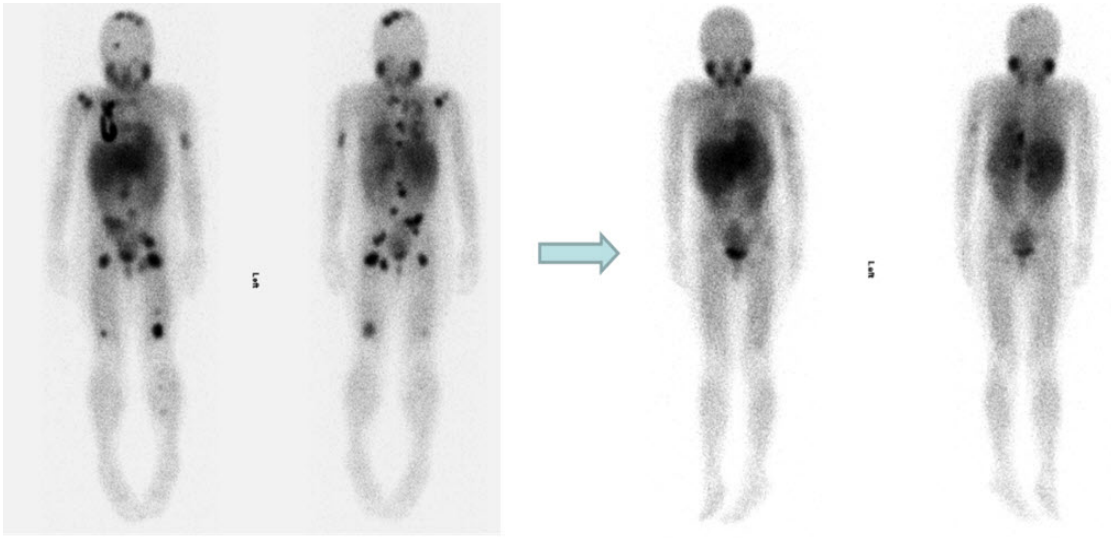
As shown in the table below, eight of 11 primary refractory patients achieved an ORR of approximately 73%, and five of 12 secondary refractory patients achieved an ORR of approximately 42%.

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

Patient group	CR/PR	
Primary refractory (n = 11)	8 (72.7)	%
Secondary refractory (n = 12)	5 (41.7)	%
All patients with non-progressive evaluable disease (n = 23)	**13 (56.5)**	**%**

CR = complete response; PR = partial response.

The scan on the left below shows multiple ^{123}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}I-MIBG. These patients, who had one to five prior relapses and therefore had a poor prognosis, showed an encouraging two-year event-free survival, or 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. In May 2018, topline results from the first 71 patients (including 29 NED patients) in this Phase 2 study were presented, which continued to show response rates at the same levels as in the dose escalation part of the study with 13 of 15 evaluable, or 87% of, primary refractory patients responding and 7 of 23 evaluable, or 30% of, secondary refractory patients responding.

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}I-MIBG scans and who were deemed to have measurable disease and be eligible for response classification by the INRC classification incorporating ^{123}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: (NED patients) To assess the impact of naxitamab and GM-CSF on PFS in patients in greater than or equal to second CR/very good partial response, or VGPR, but at high-risk of another relapse.

- In Group 2: To assess the activity of naxitamab and GM-CSF in patients who have primary refractory disease in the bone and BM by measuring response and by calculating PFS.

- In Group 3: To assess the activity of naxitamab and GM-CSF in patients who have secondary refractory disease in the bone and 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 the bone and BM after the first two cycles of naxitamab and GM-CSF have significant prognostic impact on outcome.

Safety Results

HAHA developed in 11 out of 71, or 15% of the patients. Nine out of the 11 HAHA-positive patients were previously treated with anti-GD2 antibody.

Treatment was outpatient, without unexpected toxicities.

Efficacy Results

Group 1 included 29 patients 0.9-to-17.8 (median 3.3) years post-diagnosis, 2.2-to-24.5 (median 6.3) years old, 25/29 prior-treated with ≥1 anti-G_{D2} antibody, and status-post 1 (n=18) or ≥2 (n=11) relapses; 12-month EFS was 74%.

Group 2 included 17 patients with 15 evaluable for response 5-to-19 (median 6.6) months post-diagnosis, 2.9-to-10.9 (median 5.1) years old, and 9/15 with Curie scores 7-to-23 plus marrow(+). Thirteen out of 15, or 87% of the, patients achieved CR/PR.

Group 3 included 25 patients 0.9-to-10.6 (median 3.5) years post-diagnosis, 2.6-to-23.6 (median 6.5) years old, 23/25 prior-treated with ≥1 anti-G_{D2} antibody, and status-post 1 (n=15) or 2-to-6 (n=10) relapses; 12-month PFS was 55%, and seven out of 23, or 30% of the, patients evaluable for response achieved CR/PR.

Study 201: A Phase 2 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 second quarter of 2018. We expect to enroll a total of 37 patients with recruitment ongoing in four sites.

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 ^{123}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 ^{123}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 HAHAs.

We have initiated Study 201 to form the primary basis for our planned BLA, to establish comparability of study population with Study 12-230 and to satisfy the confirmatory study and post-marketing requirements by the FDA. If the results from Study 201 fail to demonstrate comparability to the satisfaction of the FDA and other comparable regulatory authorities, this may lead to a delay in, or otherwise adversely affect, such clinical trials, including the timing of submission of the BLA.

In October 2017, the FDA issued a partial clinical hold on our IND for naxitamab. A partial clinical hold, as opposed to a full clinical hold, is a delay or suspension of only a specific part of the clinical work requested under the IND, which allows otherwise unaffected parts of the clinical work to proceed under the IND. The FDA stated that the proposed acceptance criterion for the ADCC-CD16, ADCC-CD32, and CDC assays were too wide to provide sufficient control over these attributes, which are critical for safety and efficacy. ADCC and CDC refer to antibody dependent cell-mediated cytotoxicity and complement-dependent cytotoxicity, respectively. We submitted a response to the FDA in March 2018, and met with the FDA in April 2018. Subsequently, we submitted a complete response to the partial clinical hold to the FDA in May 2018 and the partial clinical hold was removed in June 2018.

Study 16-1643: Naxitamab/GM-CSF Immunotherapy Plus Isotretinoin for Consolidation of First Remission of Patients with High-Risk Neuroblastoma: A Phase 2 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 January 2019, 37 patients had been enrolled in the study.

Patient population

In addition to satisfying certain other criteria, patients must have a 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^2/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 the 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.

Safety Results

One patient was reported with an unexpected neuropathic event. The patient suffered from short-term lower limb paralysis that resolved upon hospitalization treatment. The investigator described the event as myelitis.

Study 11-009: Phase 1 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 January 2019, 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.

Study 17-251: Pilot Study of Naxitamab, Irinotecan/Temozolomide and Sargramostim (HITS) Chemoimmunotherapy for High-Risk Neuroblastoma

Study 17-251 is a single arm pilot study in high-risk R/R NB patients with soft tissue disease. Patients will be treated with naxitamab in combination with irinotecan, temozolomide and sargramostim, or HITS. As of January 2019, 22 patients have been enrolled in the study. If the regimen is found to be acceptable, then we plan to initiate a Phase 2/3 study.

Patient population

In addition to satisfying certain other criteria, the patients must have a diagnosis of NB as defined by international criteria, including histopathology or bone marrow metastases plus high urine catecholamine levels.

High-risk NB is defined as any of the following:

- Stage 4 with MYCN amplification (any age)

- Stage 4 without MYCN amplification (greater than one and a half years of age)
- Stage 3 with MYCN amplification (unresectable; any age)
- Stage 4S with MYCN amplification (any age)

Patients must have a history of tumor progression or relapse or failure to achieve CR following standard therapy. Patients must also have evaluable disease documented after completion of prior systemic therapy.

Treatment protocol

Each cycle consists of four doses of naxitamab, five doses each of irinotecan and temozolomide and five doses of sargramostim. Irinotecan 50mg/m^2/day IV will be administered from day one through five concurrently with temozolomide 150mg/m^2/day orally. Naxitamab 2.25mg/kg IV will be administered on days two, four, eight and 10. Sargramostim 250mg/m^2/day subcutaneous will be administered from day six through 10. If patients do not experience significant toxicity they will commence a second cycle four to six weeks after the first cycle. If there is no progressive disease and patients do not experience significant toxicity they may receive combination therapy up to two years.

Primary Objective

- To evaluate the safety of HITS in patients with NB

Secondary Objective

- To evaluate tumor responses to HITS in patients with NB

Safety results

Currently, no published safety data is available for this study.

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 January 2019, 25 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 January 2019, 25 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 2 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 (^{131}I-omburtamab) or Iodine-124 (^{124}I-omburtamab). ^{131}I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB, and was granted BTD in this indication in 2017. In 2016, ^{131}I-omburtamab was granted ODD and RPDD, in each case, for the treatment of NB. 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. We expect to submit the BLA for ^{131}I-omburtamab for treatment of patients with R/R NB who have CNS/LM from NB in 2019. In addition, radiolabeled omburtamab is in Phase 1/2 clinical development for two additional rare pediatric cancers, DSRCT and DIPG. The most recent set of DSRCT data was presented in April 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 after approval of our BLA for ^{131}I-omburtamab for CNS/LM. 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 each year. 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 ^{131}I-omburtamab and preliminary indications potentially suggest promising results.

^{131}I-omburtamab and ^{124}I-omburtamab—Mechanism of Action

^{131}I-omburtamab and ^{124}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.

^{131}I-Omburtamab for the Treatment of Pediatric Central Nervous System/Leptomeningeal Metastases from Neuroblastoma

^{131}I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB, and was granted BTD in this indication in 2017. In 2016, ^{131}I-omburtamab was granted ODD and RPDD, in each case, for the treatment of NB. 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 ^{131}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. ^{131}I-omburtamab can be administered as a push injection in an outpatient setting. We expect to submit a BLA for ^{131}I-omburtamab in 2019, with a goal of receiving approval from the FDA in 2020. We plan to commercialize ^{131}I-omburtamab 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/LM from NB. 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 who relapse, 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 in the United States and Europe, combined.

^{131}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/LM from NB. 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/LM from NB. 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}I-omburtamab was first introduced in 2004, demonstrated a median OS of 5.5 months.

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

Currently, ^{131}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 January 2019, 106 patients with pediatric CNS/LM from NB had been treated with ^{131}I-omburtamab in Study 03-133. We are planning to treat an additional 32 patients in a multi-center pivotal Phase 2 trial (Study 101) with an interim pre-planned after the first 18 patients enrolled for the purposes of pharmacokinetic and dosimetry comparability between study sites using ^{131}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 for a total of 32 patients exposed. We expect to submit the BLA for ^{131}I-omburtamab for treatment of patients with CNS/LM from NB in 2019.

Study 03-133: Phase 1/2 Study of Intrathecal Radioimmunotherapy using ^{131}I-omburtamab for Central Nervous System/Leptomeningeal Neoplasms

The trial was originally designed as a Phase 1/2 clinical dose escalation study followed by cohort expansion at the recommended dose. To determine the MTD, patients received up to 70 millicurie, or mCi, ^{131}I-omburtamab as outpatients. Based on treatment result of the 50 mCi dose to treat neuroblastoma with CNS/LM metastasis and since no DLTs were experienced in the dose escalation part; the 50 mCi dose has been expanded as 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 January 2019, 106 patients with pediatric CNS/LM from NB had been treated with ^{131}I-omburtamab in Study 03-133. Of these 106 patients, 94 had been treated with 50 mCi ^{131}I-omburtamab. We expect that the safety portion of the BLA will be comprised of data from more than 200 patients treated with ^{131}I-omburtamab or ^{124}I-omburtamab across multiple indications. Study 03-133 is held open for recruitment (although enrollment into the NB cohort is put on hold after opening of clinical study 101). Hence, we plan to be able to continue to offer this experimental treatment to patients.

The table below presents a general clinical overview, including 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
Study 03-133—Patient Profile and AEs (January 2004 - August 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 (Absolute Neutrophil Counts, or ANC, hgb, platelets) (83) Gr 4 Hypersensitivity reaction (1) Gr 3 ALT/AST (5) Gr 3 Chemical Meningitis (3) Gr 4 Myelodysplastic Syndromes, or MDS/Acute Myeloid Leukemia, or 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(1)	5	22	Gr 4 MDS/AML (1)	
Total	**134**	**412**		

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

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}I-omburtamab treatment under Study 03-133. All patients had an intraventricular device implanted before enrollment in the study.

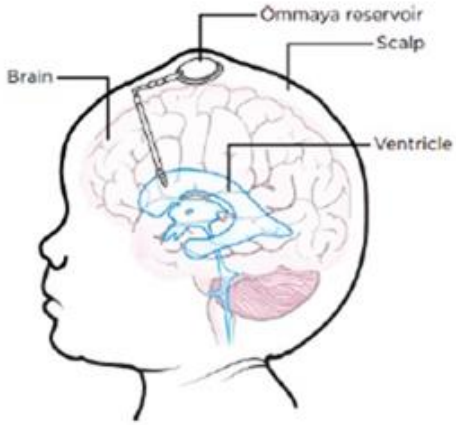
Approximately 80% of all CNS/LM from NB patients presenting at MSK since the initiation of the study were included in the study and the remaining patients were primarily excluded due to the fact that they had already received the maximum dose of previous radiotherapy to CNS, or had progressive systemic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

Treatment Protocol

Patients are treated with up to two cycles (consisting of two treatment and dosimetry doses) of ^{131}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}I-omburtamab under Study 03-133 proceeds as follows:

- Week 1: ^{131}I-omburtamab (dosimetry dose: 2-mCi imaging test dose);
- Week 2: ^{131}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 ^{131}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 ^{131}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 ^{131}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 ^{131}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 ^{131}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.

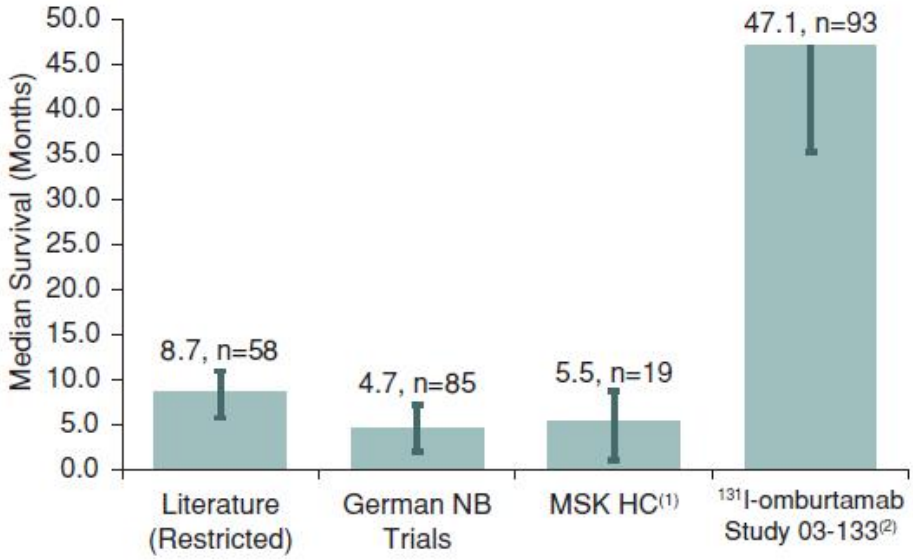
As of September 2018, 29 % of the patients had an SAE that was considered related to treatment by the investigator. The SAEs considered related by investigator were mainly in the System organ Class: investigations reflecting ^{131}I-mediated myelosuppression, which were considered related for the majority of the events. Related SAEs of vomiting were reported in five patients (3.4%), headache and meningitis chemical by four patients (2.7%) each.

Efficacy Results

Data reported as of August 2017 indicates that the median OS for the 93 patients with R/R NB who have CNS/LM from NB at relapse treated under Study 03-133 was 47 months. As of August 2017, of these 93 patients, 51, or approximately 55%, were alive. 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 ^{131}I-omburtamab in treating CNS/LM from NB.

Comparison of Median Overall Survival (Months)

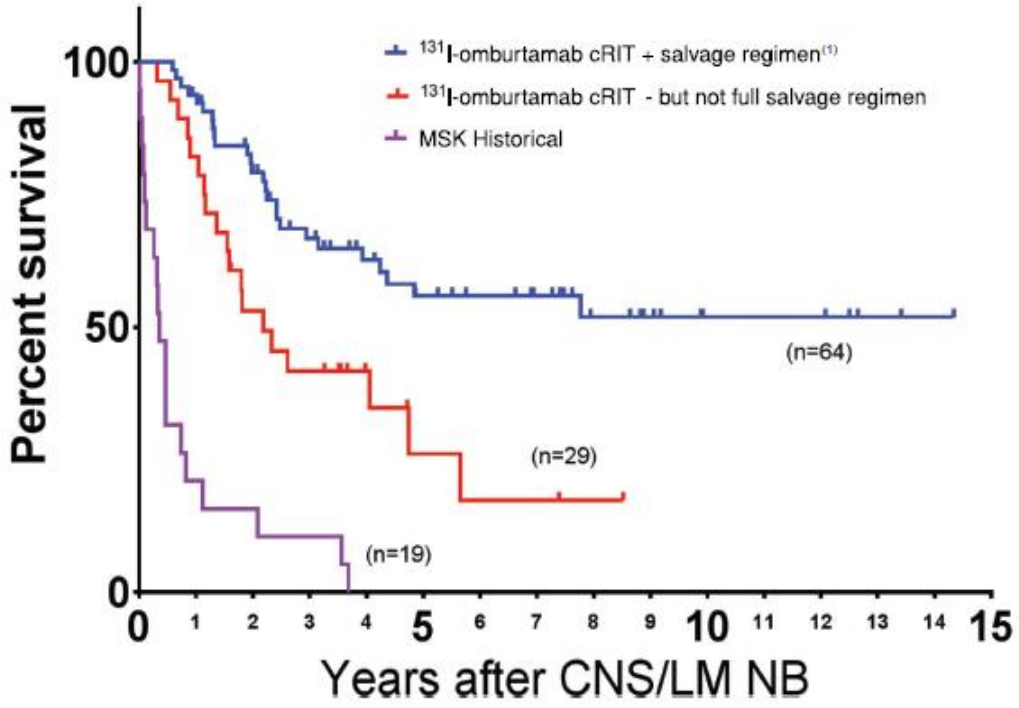


(1) MSK HC = NB patients with CNS / LM treated at MSK prior to 2003.

(2) ^{131}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 ^{131}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 ^{131}I-omburtamab.

The chart below shows the historical comparable data and median OS following the introduction of ^{131}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 ^{131}I-omburtamab remained alive at their last follow up.



(1) Salvage regimen (Kramer et al. J Neurooncology 97:409, 2012).

Study 101: A Multicenter Phase 2/3 Trial of the Efficacy and Safety of Intracerebroventricular Radioimmunotherapy using ^{131}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}I-omburtamab in pediatric patients with R/R NB who have CNS/LM from NB. Patients will receive up to two cycles of ^{131}I-omburtamab. This study commenced in the second quarter of 2018, and we plan to treat an initial 18 patients for an interim analysis for BLA submission purposes. The purpose of this part of the study is to demonstrate pharmacokinetic and dosimetry comparability between study sites using ^{131}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 at least 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 2019.

As described above 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}I-omburtamab for the treatment of pediatric patients with high-risk NB who have CNS/LM relapse.

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}I-omburtamab administration (one dosimetry dose and one treatment dose), an observation period, and post-treatment evaluations (see figure below).

One ^{131}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}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.

We have initiated Study 101 to form the primary basis for our planned BLA, to establish comparability of study population and pharmacokinetics analysis with Study 03-133 and to satisfy the confirmatory study and post-marketing requirements by the FDA. If the results from Study 101 fail to demonstrate comparability to the satisfaction of the FDA and other comparable regulatory authorities, this may lead to a delay in, or otherwise adversely affect, such clinical trials, including the timing of submission of the BLA.

^{124}I-omburtamab for the Treatment of Diffuse Intrinsic Pontine Glioma

^{124}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}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}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 convection-enhanced delivery, or CED, of ^{124}I-omburtamab in the brainstem of children with DIPG appears to be a generally feasible approach for drug delivery, based on an evaluation using distribution and pharmacokinetics. 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 the 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. We believe the current addressable market for DIPG consists of approximately 750 new pediatric DIPG patients each year in the United States and Europe, combined.

^{124}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.

^{124}I-omburtamab for Diffuse Intrinsic Pontine Glioma—Clinical Development Program

^{124}I-omburtamab is currently being evaluated in an ongoing Phase 1 clinical study (Study 11-011) for the treatment of DIPG.

Study 11-011: A Phase 1 Study of Convection-Enhanced Delivery of ^{124}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 ^{124}I-omburtamab in children with non-progressive DIPG previously treated with external beam radiation therapy. The study commenced in December 2011 and as of January 2019, 37 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}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}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}I-omburtamab administered via interstitial infusion in patients with DIPG.

Secondary Objectives

- To estimate tissue radiation doses and volumes of therapeutic distribution following ^{124}I-omburtamab interstitial infusion in the brainstem.
- To assess the toxicity profile associated with ^{124}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 approach for drug delivery in the brainstem of children with DIPG as evaluated using distribution and pharmacokinetics. As of June 2017, 28 patients had been enrolled, of which 25 patients were evaluable. Three patients were not evaluable due to partial dose delivery. Of the 25 evaluable patients, one patient experienced alanine transaminase and aspartate transaminase elevation and one patient experienced Grade 3 hemiparesis.

Multi-Center Pediatric Brain Tumor Consortium Study

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}I-omburtamab. 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}I-omburtamab for Treatment of Desmoplastic Small Round Cell Tumor

^{131}I-omburtamab is currently being evaluated in an ongoing Phase 1 clinical study (Study 09-090) for the treatment of DSRCT. In the data from 39 out of 41 patients that was presented in April 2018, 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 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. We believe the current addressable market for DSRCT consists of approximately 160 new DSRCT patients each year, representing approximately 65% of all new patients diagnosed with DSRCT in the United States and Europe, combined.

^{131}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.

^{131}I-omburtamab for Desmoplastic Small Round Cell Tumor—Clinical Development Program

Currently, ^{131}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 1 Study of Intraperitoneal Radioimmunotherapy with ^{131}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 ^{131}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 May 2018, 50 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}I-omburtamab reactive DSCRT 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}I-omburtamab, which was administered at doses ranging from 30 mCi/m^2 to 90 mCi/m^2. The expansion cohort comprised an additional 10 patients who were dosed at 80 mCi/m^2.

Primary Objective

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

Secondary Objectives

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

Safety Results

In the data from 41 patients with DSRCT presented in April 2018, no DLTs were observed and a MTD was not reached. In addition, there was no significant myelosuppression and stem cell rescue was not required. Three patients experienced Grade 3 neutropenia, three patients experienced Grade 4 neutropenia, six patients experienced Grade 3 thrombocytopenia, one patient experienced Grade 3 AST elevation and four patients experienced Grade 2 abdominal pain. We believe that the initial data from the first group of patients supports continued investigation of the benefit of ^{131}I-omburtamab in this patient population.

Non-Clinical Safety

In non-clinical studies evaluating the pharmacology and toxicology of omburtamab, no significant toxicity was 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 B7-H3 protein on the surface of cancer cells. B7-H3 expression is restricted to the liver and adrenal glands, and absent in most other human tissues, notably the brain. We believe that the lack of cross reactivity with most normal human tissues, specifically within the brain, and the localized binding of omburtamab to the surface of cancer cells that express B7-H3, makes omburtamab a viable candidate for compartmental targeted radiotherapy.

Omburtamab—DTPA Overview

We intend to leverage our expertise with omburtamab to develop product candidates for the treatment of indications associated with pediatric and large adult patient populations. We believe that our clinical experience with

^{131}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. Animal toxicity studies of ^{177}Lu-omburtamab-DTPA have been completed on current Good Laboratory Practices, or GLP, material and cGMP production has been established. 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 ^{177}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 ^{131}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 ^{177}Lu-omburtamab-DTPA could be used more frequently, thereby significantly expanding our patient population beyond children. We expect to file an IND for ^{177}Lu-omburtamab-DTPA for treatment of B7-H3 positive LM from solid tumors in 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. Based on autopsy studies, 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.

^{177}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.

^{177}Lu-Omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases—Mechanism of Action

We are developing a Lutetium-177 conjugated omburtamab with DTPA as chelator. ^{177}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 ^{131}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 ^{177}Lu-omburtamab-DTPA conjugate can be distributed conjugated ready to use. Lutathera, a Lutetium-177-DOTA conjugated somatostatin analogue peptide, has already demonstrated significant clinical efficacy in patients with progressive neuro endocrine tumors, or NETs, and is approved by the EMA, and 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 huB7-H3, a humanized version of omburtamab, for the treatment of B7-H3 positive adult solid tumors where systemic immunotherapy is needed. We expect that huB7-H3 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 novel 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 clinical product candidate, huGD2-BsAb, is a humanized anti-GD2 and anti-CD3 BsAb. The IND for this construct was cleared by the FDA December 2018.

Our second BsAb product candidate, huCD33-BsAb, is a humanized anti-CD33 and anti-CD3 BsAb. We are in pre-clinical development for our huCD33-BsAb product candidate for the treatment of huCD33-positive hermatological cancers.

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 on several MULTI-TAG candidates with MSK.

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 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.

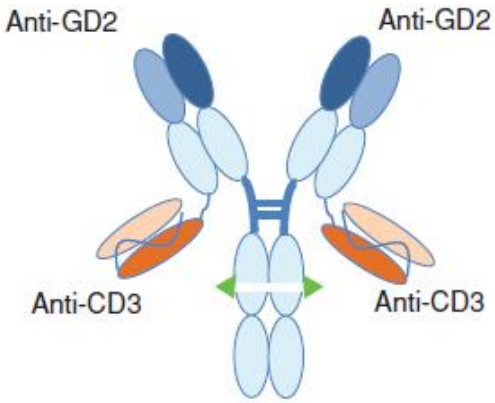
Non-IgG-like BsAbs lack a fragment crystallizable, or Fc, region, consisting instead of chemically linked variable regions and various types of multivalent single-chain variable fragments, or scFvs. One type of non-IgG-like BsAbs are BiTEs. BiTEs are relatively small and have more efficient penetration, 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 for treatment of cancer in the United States is blinatumomab, a BiTE, approved for the treatment of acute lymphocytic leukemia.

huGD2-BsAb Overview

The figure below depicts our first BsAb product candidate, huGD2-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

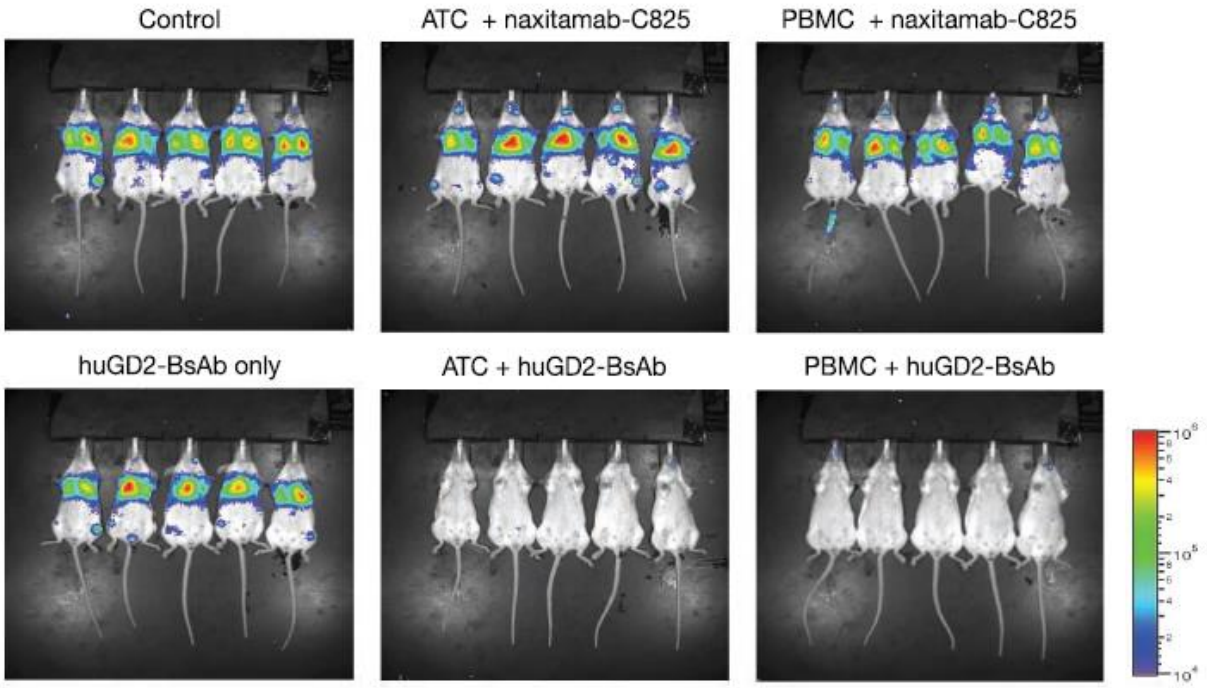


We believe that huGD2-BsAb may have several potential advantages over other BsAbs, including:

- 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; and
    - Low affinity for CD3 molecules and functional monovalency towards CD3 reduces risk of significant cytokine release.

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 (huGD2-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 huGD2-BsAb in the presence of human effector cells (ATC+huGD2-BsAb or PBMC+huGD2-BsAb) demonstrated nearly total tumor elimination. The picture below is a representative image at day 31.



On December 10, 2018, the FDA cleared the IND application for the humanized bispecific GD2 antibody, and in January 2019, a Phase 1/2 trial with our huGD2 BsAb product candidate for the treatment of refractory GD2 positive adult and pediatric solid tumors was initiated.

Study 18-034: Phase I/II study of humanized 3F8 bispecific antibody (Hu3F8-BsAb) in patients with relapsed/refractory neuroblastoma, osteosarcoma, and other GD2(+) solid tumors

Study 18-034 is a phase I/II single arm, dose escalation clinical trial of the hu3F8 bispecific antibody. Dose escalation to be performed in patients with relapsed/refractory neuroblastoma, osteosarcoma or other GD2-positive tumors. Cohort expansion will be conducted in relapsed/refractory neuroblastoma (group 1) and osteosarcoma (group 2). Up to 30 patients will enroll in Phase I and up to 64 patients will enroll in Phase II. The phase I endpoints include maximum tolerated dose, or MTD , the recommended phase II dose, or RP2D, PK, HAHA, and anti tumor activity. For phase II, the endpoint will for group 1 (RR neuroblastoma) include ORR, duration of CR and OS and for group 2 (R/R Osteosarcoma) include progression-free survival at four months, ORR, duration of CR and OS. Currently, no published safety data is available for this study.

huCD33-BsAb Overview

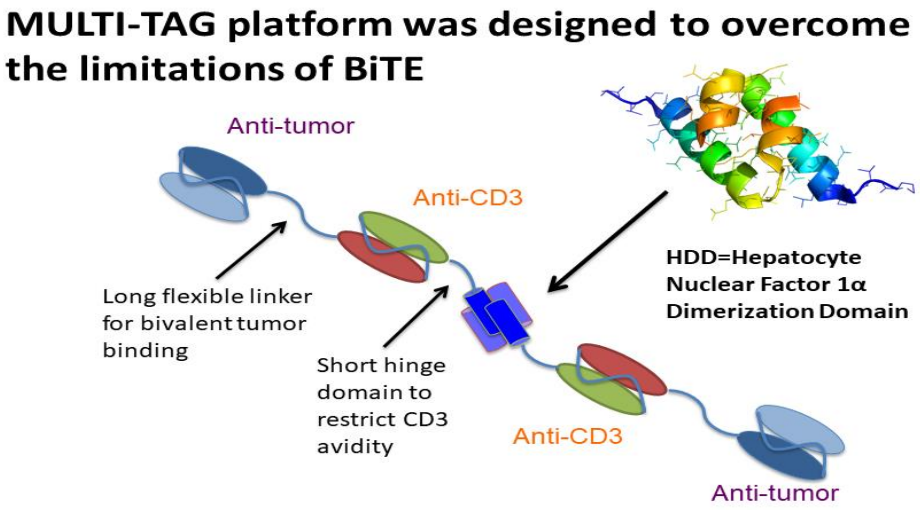
Our second BsAb product candidate, huCD33-BsAb, is a humanized anti-CD33 and anti-CD3 BsAb for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. Currently we are planning to set up GLP and cGMP production allowing for initiation of formal pre-clinical toxicology in 2019 and potential IND filing in 2020.

MULTI-TAG Technology Overview

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 the 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 on several MULTI-TAG candidates with MSK.

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 IgG1κ monoclonal antibody against GD2 expressed in Chinese Hamster Ovary, or CHO, cells. A 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, (both part of the Thermo Fisher Scientific Inc., group of companies) collectively Patheon/Thermo Fisher, 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 (now part of the Merck KgaA group of companies), or EMD/Merck, in Martillac, France, and the omburtamab drug product is manufactured by Patheon/Thermo Fisher in Ferentino, Italy.

While we believe that Patheon/Thermo Fisher and EMD/Merck 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/Thermo Fisher and/or EMD/Merck not be able to provide sufficient quantities of drug product for our planned clinical trials, we would be required to seek and then qualify 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 and 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. United Therapeutics Corporation has commercialized Unituxin (dinutuximab), an antibody against GD2, in the United States. United Therapeutics Corporation has also announced that it is developing a humanized GD2 antibody. In addition, naxitamab may face competition from dinutuximab beta, a similar antibody product against GD2 developed by Apeiron Biologics AG, or Apeiron, that was approved in Europe in May 2017 to treat high-risk NB and R/R NB. Apeiron has previously announced plans to file for registration of dinutuximab beta in the U.S. in the first quarter of 2019 in R/R NB. In October 2016, EUSA Pharma (UK) Ltd., or EUSA, announced that it had acquired global commercialization rights to dinutuximab beta, which is currently being commercialized under the name Qarziba® 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. In addition, an international patent application has been filed claiming the inventions of investigators at MSK as well as personnel of Y-mAbs Therapeutics.

As of December 31, 2018, 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 South Korean patent, one Hong Kong patent, one pending patent application in the United States with notice of allowance received, and three pending patent applications in other jurisdictions, including Europe, Canada, and India. We expect that any patents that issue in this first family will expire in June 2031. A core U.S. patent in this family is expected to expire on June 20, 2031. The second family consists of applications with composition of matter claims covering high affinity anti-GD2 antibodies, and includes one patent application in the United States granted on January 1st

2019, one pending application in Europe with intention to grant received, and eight pending patent applications in other jurisdictions, including 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 eight U.S. patents, one German patent, one Spanish patent, one French patent, one patent in Great Britain, one Italian patent, Canadian patents and one pending patent application in the United States. We expect that any patents that issue in this first family will expire between October 2021 and January 2026. A core U.S. patent in this family is expected to expire on January 19, 2026 and core patents in Germany, Spain, France, Great Britain and Italy in this family are expected to expire on March 6, 2023. 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, one Canadian patent, and one pending patent application in Europe. We expect that any patents that issue in this second family will expire in March 2028. Core patents in Canada, China, and India in this family are expected to expire on March 24, 2028.

- For our huB7-H3 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. In addition an international patent application has been filed, with MSK and the Company as applicants, claiming a method for treating a central nerve system (CNS) cancer using huB7H3, as well as 177Lu-DTPA-8H9 conjugates. We expect that any patent that issue in this family will expire in May 2038.

- Our Multimerization Technology patent portfolio, which *inter alia* relates to huGD2-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 family will expire in March 2034. A core U.S. patent in this family is expected to expire on March 25, 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 International 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.

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 assurance 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 2034. If patents are issued on our pending patent applications, the resulting patents are projected to expire on dates ranging from 2021 to 2038. 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 obtained USPTO trademark registration of the “Y-mAbs” mark. Other than Y-mAbs, 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 other than Y-mAbs. If we do not secure successfully register trademark registration for our trademarks, 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 Our 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 the MSK License and the MSK CD33 License, as of December 31, 2018, we have rights to approximately 11 issued U.S. patents, approximately five 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, all of which were recorded as long-term accrued liabilities as of December 31, 2017 and December 31, 2018, 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 the MSK License provide that MSK is entitled to receive 40% - 50% of any income generated from the sale of first such PRV, and 33% of any income generated from the sale of any subsequent PRV or the sale of other comparable incentives provided by any non-U.S. jurisdiction. Additionally, the terms of the MSK CD33 License provide that MSK is entitled to receive

25% of any income generated from the sale of any PRV or the sale of other comparable incentives provided by any non-U.S. jurisdiction.

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 the MSK License, 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 had 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 for a term of five years. The research will be conducted in accordance with a written plan and budget approved by the parties. MSK has granted us a non-exclusive, non-commercial, non-transferable, royalty-free license to use any inventions or discoveries developed by MSK within the scope of the information resulting from the project, for our internal, non-commercial research purposes. We have also been granted both a first option to negotiate an exclusive or non-exclusive commercial license to MSK’s rights in inventions developed by MSK and a first option to negotiate an exclusive license to MSK’s rights in inventions jointly developed by the parties. The term of the SRA shall continue until the earlier of (i) the completion of the activities set forth in each statement of work entered into thereunder or (ii) November 10, 2020. The SRA may be terminated for convenience by either party upon prior written notice. During 2017 and 2018 we incurred research and development expenses of $1,160,000 and $1,192,000, respectively, 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. The MDSA will expire upon the completion of activities set forth in each project description entered into thereunder; however we have the option to extend the term upon written notice to MSK. Either party may terminate the MDSA upon prior written notice in the event of an uncured material breach. During 2017 and 2018, we incurred expenses of $357,000 and $396,000, respectively, 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. During 2017 and 2018, we incurred research and development expenses of $725,000 and $3,043,000 under the MCTA.

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. During 2017 and 2018, we incurred research and development expenses of $195,000 and $325,000, respectively, under the CFSAs.

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. 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 over the royalty term, increasing to $60,000 once a patent within the licensed rights is issued, 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.

The MSK CD33 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 CD33 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 CD33 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 CD33 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.

On November 13, 2017, in connection with the MSK CD33 License, we entered into the Sponsored Research Agreement, or the CD33 SRA, with MSK pursuant to which we committed to provide aggregate research funding to MSK annually for a term of two years. The research will be conducted in accordance with a written plan and budget approved by the parties. MSK has granted us a non-exclusive, non-commercial, non-transferable, royalty-free license to use any inventions or discoveries developed by MSK within the scope of the information resulting from the research, for

our internal, non-commercial research purposes. We have also been granted both 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 the parties and our personnel. The term of the CD33 SRA shall continue until the earlier of (i) the completion of the activities set forth in each statement of work entered into thereunder or (ii) November 13, 2019. The CD33 SRA may be terminated for convenience by either party upon prior written notice. In 2017 and 2018, we incurred research and development expenses of $88,000 and $670,000 under the CD33 SRA.

On July 9, 2018, we entered into the Manufacturing Agreement with MSK's Radiochemistry and Molecular Imaging Probes Core Facility, or RMIP, pursuant to which RMIP will complete specified manufacturing activities related to ^{131}I-omburtamab in connection with our pivotal Phase 2 trials for Study 101.

MabVax Sublicense Agreement

On June 27, 2018, we entered into the MabVax Sublicense, pursuant to which MabVax granted us all of the exclusive rights granted to MabVax under the MabVax-MSK License, for a bi-valent ganglioside based vaccine intended to treat NB, or the NB vaccine. MSK originally developed the NB vaccine and licensed to MabVax as part of a portfolio of anti-cancer vaccines. In 2014, MabVax was granted ODD for the vaccine for the treatment of NB. Under the terms of the Mabvax Sublicense, we paid an upfront payment of $700,000, and we will make an additional payment of $600,000 on the first anniversary of the MabVax Sublicense. We will also be responsible for any potential downstream payment obligations to MSK related to the NB vaccine that were specified in the MabVax-MSK license agreement. This includes the obligation to pay development milestones totaling $1,400,000 and mid single-digit royalty payments to MSK. In addition, if we obtain FDA approval for the NB vaccine, then we are obligated to file with the FDA for a PRV. If the PRV is granted and subsequently sold, MabVax will receive a percentage of the proceeds from the sale thereof. The MabVax Sublicense will terminate upon the termination or expiration of the MabVax-MSK License. The MabVax License will expire, on a country-by-country basis, on the later of the expiration of (i) 10 years from the first commercial sale of the licensed product in such country or (ii) the last-to-expire valid claim covering such licensed product rights at the time of and in the country of sale. MabVax may terminate the MabVax Sublicense upon prior written notice to us in the event of our uncured material breach.

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 cGLP 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. In oncology, clinical phase I trials are normally conducted in patients, who have been

exposed to and failed/relapsed on available standard of care therapies. 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 BTD 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 of 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 many 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 transferable 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 a 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 (unless such deadline is extended). 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, 2018, we had 32 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.

Corporate Information

We were incorporated in Delaware on April 30, 2015. Our principal executive offices are located at 230 Park Avenue, Suite 3350, New York, New York 10169, and our telephone number is (646) 885-8505. Our website address is www.ymabs.com. The information contained on, or accessible through, our website is not incorporated by reference into this Form 10-K, and you should not consider any information contained in, or that can be accessed through, our website as part of this 10-K or in deciding whether to purchase our common stock.

ITEM 1A. RISK FACTORS.

Our business is subject to numerous risks. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Annual Report on Form 10 K, including our financial statements and the related notes, and in our other filings with the SEC. 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 such event, the trading price of our common stock could decline and you might 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. Our net losses were $19.2 million for the year ended December 31, 2017 and $43.3 million for the year ended December 31, 2018. As of December 31, 2018, we had an accumulated deficit of $84.8 million. We have financed our operations principally through private placements and the initial public offering 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 or 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;
- attracting, hiring, and retaining qualified personnel and
- adequately financing our operations at acceptable terms.

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. Under the MSK CD33 License, we are obligated to make potential payments of $550,000, $500,000 and $7,500,000 for clinical, regulatory and sales based milestones, respectively. 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 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. We also entered into a Sponsored Research Agreement, or the CD33 SRA, in connection with the MSK CD33 License, pursuant to which we committed to provide aggregate research funding to MSK annually for a term of two years. We entered into a Manufacturing Agreement with MSK's Radiochemistry and Molecular Imaging Probes Core Facility, or RMIP, pursuant to which RMIP will complete specified manufacturing activities related to ^{131}I-omburtamab in connection with our Phase 2 trials for Study 101. Additionally, we entered into a Sublicense Agreement, or the MabVax Sublicense, with MabVax Therapeutics Holdings, Inc., or MabVax, pursuant to which MabVax granted us all of the exclusive rights granted to MabVax under its license agreement with MSK, or the MabVax-MSK License, for a bi-valent ganglioside based vaccine intended to treat NB, or the NB vaccine. In addition to the upfront payment of $700,000 that we have made under the terms of MabVax Sublicense, we have agreed to make an additional payment of $600,000 on the first anniversary of the MabVax Sublicense. We will also be responsible for any potential downstream payment obligations to MSK related to the NB vaccine that were specified in the MabVax-MSK license agreement. This includes the obligation to pay development milestones totaling $1,400,000 and mid single-digit royalty payments to MSK.

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.

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, 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, 2018, we had approximately $147.8 million in cash and cash equivalents. We believe that our cash and cash equivalents, will be sufficient to fund our operations through the fourth quarter of 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, 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 or acquisitions, limiting our ability to conduct licensing transactions, 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.

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 may expand 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 being 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 beginning with the annual report for our fiscal year 2019. 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, 2017 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 hired finance professionals in 2018 with the plan to help mitigate the identified material weaknesses and are evaluating the implementation of additional procedures to address these material weaknesses.

We cannot assure you however that these or other measures will fully remediate the material weaknesses described above in a timely manner. We have commenced 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 the filing of this annual report on Form 10-K 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 $700.0 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 Select Market or other adverse consequences that would materially harm 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 and reimbursement 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 our products following any marketing approval, including the hiring of a direct salesforce and creation of marketing campaigns;
- a continued acceptable safety profile following any marketing approval;
- commercial acceptance by physicians and 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 and it may indicate that the market opportunity for our product candidates is smaller than we expect.

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 and broader 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, submit regulatory filings, obtain marketing approvals and delay the launch of our products, upon approval.

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 narrow the indications for use or 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, ability to raise additional financing 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, and if 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.

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 larger, 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.

In October 2017, the FDA issued a partial clinical hold on our IND for naxitamab. A partial clinical hold, as opposed to a full clinical hold, is a delay or suspension of only a specific part of the clinical work requested under the

IND, which allows otherwise unaffected parts of the clinical work to proceed under the IND. The FDA stated that the proposed acceptance criterion for the ADCC-CD16, ADCC-CD32, and CDC assays were too wide to provide sufficient control over these attributes, which are critical for safety and efficacy. ADCC and CDC refer to antibody dependent cell-mediated cytotoxicity and complement-dependent cytotoxicity, respectively. We submitted a response to the FDA in March 2018, and met with the FDA in April 2018. Subsequently, we submitted a complete response to the partial clinical hold to the FDA in May 2018 and the partial clinical hold was removed in June 2018. One or more clinical trials of our lead product candidates may be subject to additional clinical holds in the future, which may ultimately delay or otherwise adversely affect the clinical development of our lead product candidates.

In addition, we have initiated Study 101 and Study 201 to form the primary basis for our planned BLAs, to establish comparability of study population and phamacokinetics analysis with Study 03-133 and Study 12-230, respectively, and to satisfy the confirmatory study and post-marketing requirements by the FDA. If the results of these studies fail to demonstrate comparability to the satisfaction of the FDA and other comparable regulatory authorities, this may lead to a delay in, or otherwise adversely affect, such clinical trials, including the timing of submission of BLAs.

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, our interpretation of data from pre-clinical studies and clinical trials or conclude that we do not have adequate manufacturing controls or quality systems. 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 our cash and cash equivalents 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;
- availability and amount of coverage and reimbursement from government payors, managed care plans and other third-party payors; and
- the timing of competitive product introductions and other actions by competitors in the marketplace.

We currently have only a limited marketing and sales organization and have only limited 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 expand our sales and marketing capabilities or enter into agreements with third parties to sell and market such approved products.

We only have a limited sales or marketing infrastructure and have only limited 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 we must either further develop a sales and marketing organization or outsource these functions to strategic collaborators and 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 further establishing our own direct 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 further 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. United Therapeutics Corporation has commercialized Unituxin (dinutuximab), an antibody against GD2, in the United States. United Therapeutics Corporation has also announced that it is developing a humanized GD2 antibody. In addition, naxitamab may face competition from dinutuximab beta, a similar antibody product against GD2 developed by Apeiron Biologics AG, or Apeiron, that was approved in Europe in May 2017 to treat high-risk NB and R/R NB. Apeiron has previously announced plans to file for registration of dinutuximab beta in the U.S. in the first quarter of 2019. In October 2016, EUSA Pharma (UK) Ltd., or EUSA, announced that it had acquired global commercialization rights to dinutuximab beta, which is currently being commercialized under the name Qarziba® 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 contract research organizations, or CROs, or contract manufacturing organizations, 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. We currently carry $5.0 million of clinical trial insurance and expect to take out additional product liability insurance upon marketing approval of any of our product candidates. The amount of our current clinical trial and our future product liability insurance coverage that we may have, 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 most 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 datas 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, fail to do so at acceptable quality levels or prices, or fail to maintain adequate compliance with CMC guidelines of the FDA.

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 of 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, we would need to qualify any new manufacturers, our BLA submissions would need to be amended and ultimately the FDA must approve any new manufacturers. This approval would require new testing and 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;

- the risk of cross-contamination if more than one product is manufactured at our third-party manufacturer's production facilities;

- 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, and may have inadequate quality control systems.

Each of these risks could delay or prevent the completion of our clinical trials, could delay any BLA submissions, and the approval of any of our product candidates by the FDA, result in higher costs or adversely impact commercialization of our product candidates. For example, during 2018 we experienced a shortage in the supply of Iodine-131, one of the components of our ^{131}I-omburtamab product candidate, from our single source supplier. We have established a relationship with an additional supplier which we believe will be able to provide us with adequate supplies of Iodine-131. While we have not yet experienced any delays in the research and development of our ^{131}I-omburtamab product candidate to date, any such shortages in the supply of such raw materials used in the manufacture of our product candidates 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 limited 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. 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 2019 and 2020. 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 and/or sale 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. In addition, in the event of Brexit, European and worldwide economic or market conditions will be affected, which could lead to

instability in global financial markets. Brexit is likely to lead to legal uncertainty and potentially divergent national laws and regulations as the United Kingdom determines which EU laws to replace or replicate. Any of these effects of Brexit, and others we cannot anticipate, could adversely affect our business, financial condition, and results of operations.

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 BTD for 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 BTD, 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. BTD does not change the standards for product approval.

In June 2017, ^{131}I-omburtamab received BTD for the treatment of pediatric patients with R/R NB who have CNS/LM from NB. In addition, on August 20, 2018, naxitamab received BTD in combination with GM-CSF, for the treatment of high-risk NB refractory to initial therapy or with incomplete response to salvage therapy in patients greater than 12 months of age with persistent, refractory disease limited to bone marrow with or without evidence of concurrent bone involvement. We may seek BTD for some or all of our other product candidates, but we may never receive such BTD, 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 BTD, 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 CNS/LM from 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 the MSK License provide that MSK is entitled to receive 40% to 50% of any income generated from the sale of first such PRV, and 33% of any income generated from the sale of any subsequent PRV or the sale of other comparable incentives provided by any non-U.S. jurisdiction. Additionally, the terms of the MSK CD33 License provide that MSK is entitled to receive 25% of any income generated from the sale of any PRV or the sale of other comparable incentives provided by any non-U.S. jurisdiction. 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. President Trump has also 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 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 owned 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 owned 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 owned 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 2034, 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 2038, 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.

Other than our corporate name Y-mAbs, we have not yet registered our trademarks in the United States. Failure to secure such registrations could adversely affect our business.

Other than our corporate name Y-mAbs, 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 candidates 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

Our executive officers, directors and principal stockholders have ownership of a significant percentage of our stock and will be able to exercise significant influence over matters subject to stockholder approval.

As of December 31, 2018, our executive officers, directors and our stockholders, which own more than 5% of our outstanding common stock beneficially own shares representing approximately 68% of our common stock. 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 amended and restated certificate of incorporation and our amended and restated bylaws 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.

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

Our shares of common stock began trading on The NASDAQ Global Select Market on September 21, 2018. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares.

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 relies in part on the research and reports that industry or financial analysts publish about us or our business. 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.

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. Since our common stock began trading on The NASDAQ Global Select Market on September 21, 2018, our stock has traded at prices as low as $15.17 per share and as high as $31.00 per share through March 14, 2019. As a result of this volatility, you may not be able to sell your common stock at or above the price your paid for it.

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 revenues and 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;
- our ability to accurately forecast demand for our products, actual or anticipated changes in forecasts of financial performance, or changes in 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 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 $700.0 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, 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. 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 incur increased costs as a result of operating as a public company, and our management is 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 devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, these rules and regulations 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. The Company has performed an analysis of its Section 382 ownership changes through December 31, 2018. Due to the large annual limitation, the Company believes that it is more likely than not that none of the net operating loss carryforwards will expire as a result of the limitation from the ownership change under Section 382.

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.

Future sales of common stock by stockholders may have an adverse effect on the then prevailing market price of our common stock.

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.

Sales of our common stock may be made by holders of our public float or by holders of restricted securities in compliance with the provisions of Rule 144 of the Securities Act of 1933, or the Securities Act.

There were 34,193,666 shares of common stock outstanding as of March 14, 2019. Of these shares of our common stock, 6,900,000 shares sold in our initial public offering in 2018 are freely tradable, without restriction, in the public market. Almost all of the holders of the remaining 27,293,666 shares of common stock or approximately 80 % of our total outstanding common stock as of March 14, 2019 have the right, 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.

Also, in general under Rule 144, a non-affiliated person who has satisfied a six-month holding period in a company registered under the Exchange Act, as amended, may, sell their restricted common stock without volume limitation, so long as the issuer is current with all reports under the Exchange Act in order for there to be adequate common public information. Affiliated persons may also sell their common shares held for at least six months, but affiliated persons will be required to meet certain other requirements, including manner of sale, notice requirements and volume limitations. Non-affiliated persons who hold their common shares for at least one year will be able to sell their common stock without the need for there to be current public information in the hands of the public. Future sales of shares of our public float or by restricted common stock made in compliance with Rule 144 may have an adverse effect on the then prevailing market price, if any, of our common stock.

Our amended and restated certificate of incorporation 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 amended and restated certificate of incorporation 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) is 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.

ITEM 1B. UNRESOLVED STAFF COMMENTS.

None.

ITEM 2. PROPERTIES.

Our corporate headquarters are located in New York, New York, where we currently lease 4,312 square feet pursuant to a lease agreement dated as of January 10, 2018, which expires five years from the date we first began to

occupy the premises. In addition, we lease 4,548 square feet of combined office and laboratory space located in Nutley, New Jersey pursuant to a lease agreement dated as of February 11, 2019, which expires on February 10, 2022.

Our wholly owned Danish subsidiary, Y-mAbs Therapeutics A/S, leases approximately 15,087 square feet of office space in Hørsholm, Denmark pursuant to a lease agreement dated February 2, 2018 as amended on November 19, 2018 and February 22, 2019. The lease may be terminated by us with nine months notice made no earlier than August 2022. The landlord may not terminate the lease until April 2024.

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.

ITEM 3. 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.

ITEM 4. MINE SAFETY DISCLOSURES.

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES.

Certain Information Regarding the Trading of Our Common Stock

Our common stock trades under the symbol "YMAB" on the NASDAQ Global Select Market and has been publicly traded since September 21, 2018. Prior to this time, there was no public market for our common stock.

On March 14, 2019, the last reported sale price for our common stock on the NASDAQ Global Select Market was $21.38 per share.

Holders of Our Common Stock

As of March 14, 2019, there were 55 holders of record of shares of our common stock. This number does not include stockholders for whom shares are held in "nominee" or "street" name.

Dividends

We have never declared or paid any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings for use in the operation of our business and do not anticipate paying any dividends on our common stock in the foreseeable future. Any future determination to declare dividends will be made at the discretion of our board of directors and will depend on our financial condition, operating results, capital requirements, general business conditions and other factors that our board of directors may deem relevant.

Securities authorized for issuance under equity compensation plans

Information about our equity compensation plans will be included in our definitive proxy statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders and is incorporated herein by reference.

Unregistered Sales of Equity Securities and Use of Proceeds

Recent Sales of Unregistered Equity Securities

During the period covered by this Annual Report on Form 10-K, we have issued the following securities that were not registered under the Securities Act:

• Between January 1, 2018 and December 31, 2018, we issued to certain of our employees and directors and a shareholder, options to purchase an aggregate of 1,138,873 shares of our common stock at a weighted-average exercise price of $16.56 per share.

• On August 20, 2018, we issued 448,000 shares pursuant to stock grant agreements entered into in August 2015 with two physicians who were involved in the development of technology licensed from MSK in consideration for their prior services.

• On September 21, 2018, we issued an additional 96,000 shares pursuant to one of the stock grant agreements entered into in August 2015 in conjunction with the Company's initial public offering due to accelerated vesting in such stock grant agreement.

We deemed the issuances in the paragraphs above to be exempt from registration under the Securities Act either in reliance on Rule 701 of the Securities Act as sales and offers under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701, or in reliance on Section 4(a)(2), as transactions by an issuer not involving a public offering. Each of the recipients of securities either received adequate information about our company or had access, through employment or other relationships, to such information. No underwriters were involved in the foregoing issuances of securities.

Use of Proceeds from Initial Public Offering

On September 25, 2018, we completed the initial public offering of our common stock, or IPO pursuant to which we issued and sold 6,900,000 shares of our common stock at a price to the public of $16.00 per share which included the exercise in full of the underwriters' option to purchase additional shares.

The offer and sale of all of the shares in our initial public offering were registered under the Securities Act pursuant to a registration statement on Form S-1, which was declared effective by the SEC on September 10, 2018, and a registration statement on Form S-1MEF, which was automatically effective upon filing with the SEC on September 20, 2018. Following the sale of the shares in connection with the closing of our initial public offering, the offering terminated. Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC acted as joint book-running managers for the offering. Canaccord Genuity LLC acted as lead manager for the offering and BTIG, LLC acted as co-manager for the offering.

We received aggregate gross proceeds from our initial public offering of $110.4 million, or aggregate net proceeds of approximately $99.8 million after deducting underwriting discounts and commissions and offering expenses. None of the underwriting discounts and commissions or offering expenses were incurred or paid, directly or indirectly, to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any of our affiliates, and we have not used any of the net proceeds from the offering to make payments, directly or indirectly, to any such persons. We have invested the net proceeds from the offering in money market accounts. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 24, 2018.

As of December 31, 2018, we have not used the net proceeds from our initial public offering.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

ITEM 6. SELECTED 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, 2017 and 2018 and the consolidated balance sheet data as of December 31, 2017 and December 31, 2018 from our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K. 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 Annual Report on Form 10-K.

	For the year ended December 31,	
	2018	2017
	(in thousands, except per share data)	
Consolidated Statement of Operations Data:		
Revenue	$ —	$ —
Operating expenses:		
Research and development	34,269	14,307
General and administrative	8,961	4,937
Total operating expenses	43,230	19,244
Loss from operations	(43,230)	(19,244)
Interest and other income (expense)	(44)	83
Net loss	$ (43,274)	$ (19,161)
Net loss attributable to common stockholders	$ (43,274)	$ (19,161)
Net loss per share attributable to common stockholders—basic and diluted[1]	$ (1.50)	$ (0.99)
Weighted-average common shares outstanding used in computing net loss per share attributable to common stockholders—basic and diluted[1]	28,772,384	19,397,506

(1) See Note 4 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K 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.

	December 31, 2018	December 31, 2017
	(in thousands)	
Consolidated Balance Sheet Data:		
Cash and cash equivalents	$ 147,840	$ 90,483
Working capital[1]	142,409	83,430
Total assets	151,924	92,127
Total liabilities	11,397	9,975
Accumulated deficit	(84,835)	(41,561)
Total stockholders' equity	140,527	82,152

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

ITEM 7. 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 Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, 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 Annual Report on Form 10-K, 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 2019, with a goal of receiving approval by the FDA in 2020. 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 huB7-H3, a humanized version of omburtamab, each targeting indications with large adult patient populations. In addition, we have initiated a Phase I trial with our huGD2 BsAb product candidate for the treatment of refractory GD2 positive adult and pediatric solid tumours, thereby addressing large patient populations. We are also advancing a pipeline of novel BsAbs through late pre-clinical development, including our huCD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. We believe our BsAbs have the potential to result in improved tumor-binding, longer serum half-life 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 on April 30, 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 and the proceeds of our initial public offering. On September 25, 2018, we completed the initial public offering, or IPO, of our common stock pursuant to which we issued and sold 6,900,000 shares at a price of $16.00 per share which included the exercise in full of the underwriters' option to purchase additional shares for gross proceeds of approximately $110.4 million, before deducting underwriting discounts and commissions and estimated offering expenses. We have received aggregate gross proceeds of $230.0 million through December 31, 2018 from the sale and issuance of our common stock.

As of December 31, 2018, we had an accumulated deficit of $84.8 million. Our net losses were $43.3 million for the year ended December 31, 2018 and $19.2 million for the year ended December 31, 2017. 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 will be sufficient to fund our operations through the fourth quarter of 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 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 the SRA, the two CFSAs, the MCTA, and the 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 non-revenue related 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 and regulatory approval 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 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 timing of our BLA submissions and their acceptance;
- 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, 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 Annual Report on Form 10-K, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

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.

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.

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 to employees and directors 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

Prior to our IPO, the estimated fair value of our common stock was determined by our board of directors as of the date of each award grant, with input from management, considering our then most recently completed or ongoing private placement activities and then most recently available third-party valuation of our common stock. Third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants’ Accounting and Valuation Guide, Valuation of Privately-Held-Company Equity Securities issued as Compensation. This process 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, October 21, 2016 and August 22, 2016; $8.50 per share as of December 14, 2016; $9.35 per share as of September 13, 2017 and December 5, 2017; $11.16 per share as of April 24, 2018; and $13.11 as of July 10, 2018. 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 a sale of our company in light of prevailing market conditions; and
- an analysis of initial public offerings and the market performance of similar companies in the biopharmaceutical industry.

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

Since the closing of our IPO, our board of directors has determined the per share fair value of our common stock based on the closing price of our common stock as reported by the NASDAQ Global Select Market on the date of grant.

Stock Options Granted

All options to purchase shares of our common stock are granted with an exercise price per share equal to or greater than the estimated fair value per share of our common stock on the date of grant, based on the information known to us on the date of grant. The following table sets forth by grant date the number of shares of common stock subject to options granted from 2016 to 2018, 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:

Grant Date	Type of Award	Number of Shares	Per Share Exercise Price	Estimated Fair Value Per Share on Grant Date
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
September 13, 2017	Option	40,000	$ 9.35	$ 5.58
December 5, 2017	Option	20,000	$ 9.35	$ 5.62
April 24, 2018	Option	520,373	$ 11.16	$ 6.42 - $8.09
July 10, 2018	Option	60,000	$ 13.11	$ 7.54
December 11, 2018	Option	558,500	$ 21.97	$ 12.49 - $12.70

We expect the amount of share-based compensation expense recognized for stock options to increase for future awards in future periods due to the potential increase in both the value of our common stock and the size of our company in terms of headcount.

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.

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, 2018	Year Ended December 31, 2017
Risk-free interest rate	2.89 %	2.10 %
Expected term (in years)	6.3	7.0
Expected volatility	57.8 %	58.9 %
Expected dividend yield	— %	— %

The assumptions that the Company used to determine the fair value of the stock options granted to non-employees were as follows, presented on a weighted average basis:

	Year ended December 31, 2018
Risk-free interest rate	3.00 %
Expected term (in years)	10.0
Expected volatility	62.7 %
Expected dividend yield	— %

- 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 Term: 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. 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, 2018 and 2017

The following table summarizes our results of operations for the years ended December 31, 2018 and 2017:

	Year Ended December 31,		
	2018	2017	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 34,269	$ 14,307	$ 19,962
General and administrative	8,961	4,937	4,024
Total operating expenses	43,230	19,244	23,986
Loss from operations	(43,230)	(19,244)	(23,986)
Interest and other income (expense)	(44)	83	(127)
Net loss	$ (43,274)	$ (19,161)	$ (24,113)

Research and Development Expenses

We do not record our research and development expenses on a program-by-program or on a product-by-product basis as they primarily relate to personnel, research, manufacturing, license fees, non-cash expense in connection with equity issuances to strategic partner and consumable costs, which are simultaneously deployed across multiple projects under development. These costs are included in the table below.

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Outsourced manufacturing	$ 13,389	$ 5,931
License agreements (milestone and royalty obligations)	1,300	700
Clinical trials	3,398	633
Outsourced research and supplies	10,070	5,427
Personnel costs	3,353	449
Professional and consulting fees	1,081	521
Stock based compensation	861	609
Other	817	37
	$ 34,269	$ 14,307

Research and development expenses increased by $20.0 million, from $14.3 million for the year ended December 31, 2017, to $34.3 million for the year ended December 31, 2018. This was primarily due to a $7.5 million increase in manufacturing services and a $4.6 million increase in outsourced services and supplies, primarily obtained from MSK and CROs for our lead product candidates, naxitamab and omburtamab. Other clinical trial costs increased by $2.8 million for the year ended December 31, 2018. Employee-related costs including salary, benefits and non-cash stock-based compensation for personnel related to our research activities, increased by $3.2 million for the year ended December 31, 2018. Professional and consulting fees increased by $0.6 million for the year ended December 31, 2018.

General and Administrative Expenses

General and administrative expenses increased by $4.0 million from $5.0 million for the year ended December 31, 2017 to $9.0 million for the year ended December 31, 2018. The increase in general and administrative expenses was primarily attributable to a $1.8 million increase in employee-related costs, including salary, benefits and non-cash stock-based compensation for personnel related to our business activities. In addition, leasehold expenses

increased by $0.4 million and fees for auditors, legal advice and other consultancy services increased by $0.7 million for the year ended December 31, 2018.

Interest and Other Income (Expense)

Other Income for the year ended December 31, 2017 were $83,000 as compared to Other Expenses of ($44,000) for the year ended December 31, 2018. Our interest income has not been significant due to low interest earned on cash 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, 2018 primarily through gross proceeds of $230.0 million from the sale of our common stock. As of December 31, 2018, we had cash and cash equivalents of $147.8 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, 2018 and December 31, 2017:

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Cash used in operating activities	$ (41,229)	$ (15,870)
Cash used in investing activities	(234)	—
Cash provided by financing activities	98,763	89,586
Effect of exchange rates on cash and cash equivalents	56	(104)
Net increase in cash and cash equivalents	$ 57,356	$ 73,612

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 $41.2 million during the year ended December 31, 2018, as compared to $15.9 million during the year ended December 31, 2017. The $25.3 million increase in net cash used in operations was primarily due to an increase in our net loss of $24.1 million for the year ended December 31, 2018. This increase was primarily due to an increase in our operating expenses in connection with the development of our lead product candidates, naxitamab and omburtamab, and the expansion of our other business activities. Non-cash expenses included stock-based compensation to employees and non-employees, which increased by $1.4 million. Adjustment of working capital reflects changes in other current assets, accrued expenses, accounts payable, and other non-current liabilities of $2.7 million for the year ended December 31, 2017, as compared to $(0.1) million the year ended December 31, 2018.

Net Cash Used in Investing Activities

Net cash used in investing activities was $0.2 million for the year ended December 31, 2018, as compared to no investment activities for the year ended December 31, 2017. The $0.2 million increase in net cash used in investing activities relates to investment in furniture for our new office facilities.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $98.8 million during the year ended December 31, 2018, as compared to $89.6 million during the year ended December 31, 2017. The cash provided by financing activities was attributable to net proceeds of $99.8 million related to the issuance of common stock in the Company's IPO in the year ended December 31, 2018, which exceed the $89.8 million net proceeds related to the issuance of common stock in a private placement 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, 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 our existing cash and cash equivalents as of December 31, 2018, will enable us to fund our operating expenses and capital expenditure requirements through the fourth quarter of 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

Contractual obligations as of December 31, 2018 are related to payments of operating leases for our corporate headquarters in New York, New York and our office space in Hørsholm, Denmark. Our obligations and commitments are disclosed in the contractual obligations table below:

	Payments Due By Period				
	Total	Less Than 1 Year	1 to 3 Years	4 to 5 Years	More Than 5 Years
Operating Lease Commitments	$2,883,796	$510,071	$1,231,812	$1,077,772	$64,141
Total	$2,883,796	$510,071	$1,231,812	$1,077,772	$64,141

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.

We have entered into two license agreements and certain other agreements with MSK. The license agreements are further described below as the MSK License and the MSK CD33 License.

Under the MSK License and 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. Under the MSK License, we are also obligated to pay annual minimum royalties of $80,000 over the royalty term, starting in 2020. Under the MSK CD33 License, we are obligated to pay annual minimum royalties of $40,000 over the royalty term beginning in 2027, increasing to $60,000 once a patent within the licensed rights is issued. These amounts are non-refundable but are creditable against royalty payments otherwise due under the respective agreements. Total expensed minimum royalty payments in 2016 under the MSK License were $1,200,000, upon determination that the payment of such minimum royalties was probable and the amount was estimable in 2016. We are also obligated to pay MSK certain clinical, regulatory and sales-based milestone payments under the MSK License and MSK CD33 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. Total clinical, regulatory and sales based milestones potentially due under the MSK License are $2,450,000, $9,000,000 and $20,000,000, respectively. In addition, under the MSK CD33 License, we are obligated to make potential payments of $550,000, $500,000 and $7,500,000 for clinical, regulatory and sales based milestones, respectively. We record milestones in the period in which the contingent liability is probable and the amount is reasonably estimable. Research and development is inherently uncertain and, should such research and development fail, the MSK License and MSK CD33 License are cancelable at our option. We have also considered the development risk and each party's termination rights under the two license agreements when considering whether any contingent payments, certain of which also contain time-based payment requirements, were probable. In addition, to the extent we enter into sublicense arrangements, we are obligated 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 achievement of certain clinical milestones. To date, we have not entered into any sublicenses related to the MSK

License or the MSK CD33 License. Our failure to meet certain conditions under such arrangements could cause the related license to such licensed product to be canceled and could result in termination of the entire respective arrangement with MSK. In addition, we may terminate the MSK License or the MSK CD33 License with prior written notice to MSK. Total milestones expensed in 2017 under the MSK License were $150,000, all of which related to clinical milestones, which become due either based upon the passage of time or achievement of the related milestone activities. Total milestones expensed in 2017 under the MSK CD33 License was $550,000, all of which related to clinical milestones, which become due either based upon the passage of time or achievement of the related milestone activities. No milestones were expensed in 2018 under the MSK License and the MSK CD33 License.

On June 27, 2018, we entered into the Sublicense Agreement, or the MabVax Sublicense, with MabVax Therapeutics Holdings, Inc., or MabVax, pursuant to which MabVax granted us all of the exclusive rights granted to MabVax under its license agreement with MSK, or the MabVax-MSK License, for a bi-valent ganglioside based vaccine intended to treat NB, or the NB vaccine. MSK originally developed the NB vaccine and licensed to MabVax as part of a portfolio of anti-cancer vaccines. Under the terms of the MabVax Sublicense, we paid MabVax an upfront payment of $700,000, and, as we have decided to move forward with the development of the vaccine, we have agreed to make an additional payment of $600,000 on the first anniversary of the MabVax Sublicense. We will also be responsible for any potential downstream payment obligations to MSK related to the NB vaccine that were specified in the MabVax-MSK license agreement. This includes the obligation to pay development milestones totaling $1,400,000 and mid single-digit royalty payments to MSK. In addition, if we obtain FDA approval for the NB vaccine, then we are obligated to file with the FDA for a PRV. If the PRV is granted and subsequently sold, MabVax will receive a percentage of the proceeds from the sale thereof. The MabVax Sublicense will terminate upon the termination or expiration of the MabVax-MSK License. Total milestones expensed in 2018 under the MabVax License were $1,300,000, all of which relate to the upfront payment and payment for the first anniversary of the MabVax 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 our initial public 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 and qualify as a smaller reporting company we expect that:

- we will present 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 as long as we continue to qualify as a smaller reporting company;

- 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 our initial public 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.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK.

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2018 and December 31, 2017, we had cash and cash equivalents of $147.8 million and $90.5 million, respectively, maintained primarily with financial institutions in federally insured accounts. 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, 2018 and December 31, 2017, we had cash and cash equivalents denominated in DKK of $0.8 million and $0.6 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.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Shareholders of Y-mAbs Therapeutics, Inc.:

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Y-mAbs Therapeutics, Inc. and its subsidiary (the “Company”) as of December 31, 2018 and 2017, and the related consolidated statements of comprehensive loss, changes in stockholders’ equity and cash flows for each of the two years in the period ended December 31, 2018, including the related notes (collectively referred to as the “consolidated financial statements”). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2018 in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on the Company’s consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial

statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

Florham Park, New Jersey
March 22, 2019

We have served as the Company's auditor since 2017.

Y-MABS THERAPEUTICS, INC.

Consolidated Balance Sheets

(in thousands, except share data)

	December 31, 2018	December 31, 2017
ASSETS		
CURRENT ASSETS		
Cash and cash equivalents	$ 147,840	$ 90,483
Restricted cash	31	32
Other current assets	3,661	840
Total current assets	151,532	91,355
Property and equipment, net	205	—
Deferred offering costs	—	772
Other assets	187	—
TOTAL ASSETS	$ 151,924	$ 92,127
LIABILITIES AND STOCKHOLDERS' EQUITY		
LIABILITIES		
Accounts payable	$ 5,872	$ 5,909
Accrued liabilities	3,251	2,016
Total current liabilities	9,123	7,925
Accrued milestone and royalty payments	2,050	2,050
Other liabilities	224	—
TOTAL LIABILITIES	11,397	9,975
Commitments and contingencies (Note 6)		
STOCKHOLDERS' EQUITY		
Preferred stock, $0.0001 par value, 5,500,000 shares authorized at December 31, 2018 and December 31, 2017; none issued at December 31, 2018 and December 31, 2017	—	—
Common stock, $0.0001 par value, 100,000,000 and 50,000,000 shares authorized at December 31, 2018 and December 31, 2017, respectively; 34,193,666 and 26,749,666 shares issued at December 31, 2018, and December 31, 2017, respectively	3	3
Additional paid in capital	225,352	123,879
Accumulated other comprehensive income/(loss)	7	(169)
Accumulated deficit	(84,835)	(41,561)
TOTAL STOCKHOLDERS' EQUITY	140,527	82,152
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 151,924	$ 92,127

The accompanying notes are an integral part of the consolidated financial statements

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Comprehensive Loss

(In thousands, except share and per share data)

	For the year ended December 31,	
	2018	2017
OPERATING EXPENSES		
Research and development	$ 34,269	$ 14,307
General and administrative	8,961	4,937
Total operating expenses	43,230	19,244
Loss from operations	(43,230)	(19,244)
OTHER INCOME/(EXPENSES)		
Interest and other income/(expenses)	(44)	83
NET LOSS	$ (43,274)	$ (19,161)
Other comprehensive income/(loss)		
Foreign currency translation	175	(199)
COMPREHENSIVE LOSS	$ (43,099)	$ (19,360)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.50)	$ (0.99)
Weighted average common shares outstanding, basic and diluted	28,772,384	19,397,506

The accompanying notes are an integral part of the consolidated financial statements

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Changes in Stockholders' Equity

(In thousands, except share data)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive (Loss)/Income	Accumulated Deficit	Stockholders' Equity
	Shares	Amount				
Balance December 31, 2016	16,552,884	$ 2	$ 34,429	$ 30	$ (22,400)	$ 12,061
Issuance of common stock to investors, net of issuance costs	9,748,782	1	88,841	—	—	88,842
Issuance of common stock to nonemployees	448,000	—	—	—	—	—
Stock-based compensation expense	—	—	609	—	—	609
Foreign currency translation	—	—	—	(199)	—	(199)
Net loss	—	—	—	—	(19,161)	(19,161)
Balance December 31, 2017	26,749,666	$ 3	$ 123,879	$ (169)	$ (41,561)	$ 82,152
Issuance of common stock in initial public offering, net of issuance costs	6,900,000	—	99,507	—	—	99,507
Issuance of common stock to nonemployees	544,000	—	—	—	—	—
Stock-based compensation expense	—	—	1,966	—	—	1,966
Foreign currency translation	—	—	—	176	—	176
Net loss	—	—	—	—	(43,274)	(43,274)
Balance December 31, 2018	34,193,666	$ 3	$ 225,352	$ 7	$ (84,835)	$ 140,527

The accompanying notes are an integral part of the consolidated financial statements

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Cash Flows

(In thousands)

	For the year ended December 31,	
	2018	2017
CASH FLOWS FROM OPERATING ACTIVITIES		
Net loss	$ (43,274)	$ (19,161)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	29	—
Stock-based compensation	1,966	609
Foreign currency transactions	97	(63)
Changes in assets and liabilities:		
Other current assets	(2,821)	(482)
Other assets	(187)	—
Accounts payable	1,261	2,385
Accrued liabilities and other	1,700	842
NET CASH USED IN OPERATING ACTIVITIES	(41,229)	(15,870)
CASH FLOWS FROM INVESTING ACTIVITIES		
Purchase of property and equipment	(234)	—
NET CASH USED IN INVESTING ACTIVITIES	(234)	—
CASH FLOWS FROM FINANCING ACTIVITIES		
Proceeds from issuance of common stock, net of issuance costs	99,765	89,844
Payment of offering costs	(1,002)	(258)
NET CASH PROVIDED BY FINANCING ACTIVITIES	98,763	89,586
Effect of exchange rates on cash and cash equivalents	56	(104)
NET INCREASE IN CASH AND CASH EQUIVALENTS	57,356	73,612
Cash, cash equivalents and restricted cash at the beginning of period	90,515	16,903
Cash, cash equivalents and restricted cash at the end of period	$ 147,871	$ 90,515
SUPPLEMENTAL DISCLOSURE OF NONCASH FINANCING ACTIVITIES		
Common stock issuance cost in accounts payable	$ —	$ 1,002
Deferred offering costs included in other assets and accounts payable and accrued liabilities and other	$ —	$ 514

The accompanying notes are an integral part of the consolidated financial statements

Y-MABS THERAPEUTICS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1—ORGANIZATION AND DESCRIPTION OF BUSINESS

Y-mAbs Therapeutics, Inc. ("we," "us," "our," the "Company," or "Y-mAbs") is 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 entered into a worldwide license and research collaboration agreement (the "MSK License Agreement") with Memorial Sloan-Kettering Cancer Center ("MSK") our strategic partner, under which we have obtained the exclusive rights to MSK's rights to two clinical stage antibody-based product development programs for the treatment of neuroblastoma and other oncology indications. The MSK License Agreement 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 payer 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 $84.8 million as of December 31, 2018 and $41.6 million as of December 31, 2017. Through December 31, 2018, the Company has funded its operations through proceeds from sales of shares of its common stock, including its initial public offering, or IPO, in September 2018. As of December 31, 2018, the Company had cash and cash equivalents of $147.8 million, and as of December 31, 2017 the Company had cash and cash equivalents of $90.5 million. As of the issuance date of the annual financial statements for the year ended December 31, 2018, the Company expects that its cash and cash equivalents at December 31, 2018 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 accompanying consolidated financial statements reflect 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.

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.

Restricted cash represents a bank account with funds to cover the Company's corporate credit card availability.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization expense is recognized using the straight-line method over the estimated useful life of each asset as follows:

	Estimated Useful Life
Furniture and fixtures	5 years
Leasehold improvements	Shorter of life of lease or 15 years

Depreciation and amortization expense on property and equipment was $29,000 and $0 for the years ended December 31, 2018 and 2017, respectively.

Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance are charged to expense as incurred.

Impairment of Long-Lived Assets

ASC 360, Property, Plant and Equipment, addresses the financial accounting and reporting for impairment or disposal of long-lived assets. The Company reviews the recorded values of long-lived assets for impairment whenever events or changes in business circumstance indicate that the carrying amount of an asset or group of assets may not be fully recoverable.

Deferred Offering Costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs within other assets until such financings are consummated. After consummation of the equity financing, these costs are recorded in shareholders' equity as a

reduction of proceeds generated as a result of the offering. Should the planned equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the consolidated statement of comprehensive loss. The Company did not record any deferred offering costs as of December 31, 2018. The Company recorded deferred offering costs of $0.8 million as of December 31, 2017. Accounts payable and accrued liabilities at December 31, 2017 included $0.2 million of deferred offering costs. These deferred operating costs were reclassified to shareholders' equity upon the successful completion of the IPO during the year ended December 31, 2018.

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.

In accordance with guidance issued by Financial Accounting Standards Board ("FASB"), companies should make and disclose a policy election as to whether they will recognize deferred taxes for basis differences expected to reverse as Global Intangible Low-Taxed Income ("GILTI") or whether they will account for GILTI as period costs if and when incurred. The Company has elected to recognize the resulting tax with respect to the GILTI provision as a period cost. No costs were incurred by the Company through December 31, 2018 as a result of GILTI.

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 and development 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 its license agreement with MSK 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 $34.3 million and $14.3 million for the years ended December 31, 2018 and 2017, respectively.

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.

Foreign Currency

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, and totaled $(97,000) and $63,000 for the years ended December 31, 2018 and 2017, respectively.

Recently Issued Accounting Pronouncements - Adopted

In March 2018, the FASB issued Accounting Standards Update No. 2018-05 ("ASU 2018-05"), Amendments to SEC Paragraphs Pursuant to SEC Staff Accounting Bulletin (SAB) No. 118. The ASU adds seven paragraphs to ASC 740, Income Taxes, that contain SEC guidance related to SAB 118 (codified as SEC SAB Topic 5.EE, "Income Tax Accounting Implications of the Tax Cuts and Jobs Act"), which provides guidance for companies that are not able to complete their accounting for the income tax effects of the Tax Cuts and Jobs Act in the period of enactment which is the period that includes December 22, 2017. The measurement period should not extend beyond one year from the enactment date. The Company completed its accounting for the income tax effects

for the year ended December 31, 2018. See Note 10 for a discussion of the impact of the guidance on the Company's consolidated financial statements.

In May 2017, the FASB issued Accounting Standards Update No. 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 adopted ASU 2017-09 on January 1, 2018 and will account for any modifications in accordance with ASU 2017-09 subsequent to the effective date. The adoption of this standard on January 1, 2018 did not have a material impact on our consolidated financial statements and related disclosures.

In November 2016, the FASB issued Accounting Standards Update No. 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 adoption of this standard on January 1, 2018 did not have a material impact on our consolidated financial statements and related disclosures.

In October 2016, the FASB issued Accounting Standards Update No. 2016-16 ("ASU 2016-16"), Intra-Entity Transfers of Assets Other than Inventory, as part of its simplification initiative. Under the new guidance, an entity should recognize the income tax consequences of an intra-entity transfer of an asset other than inventory when the transfer occurs. Consequently, the amendments in this Update eliminate the exception for an intraentity transfer of an asset other than inventory. ASU 2016-16 is effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. The adoption of this standard on January 1, 2018 did not have a material impact on our consolidated financial statements and related disclosures.

In August 2016, the FASB issued Accounting Standards Update No. 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. The adoption of this standard on January 1, 2018 did not have a material impact on our consolidated financial statements and related disclosures.

In March 2016, the FASB issued Accounting Standards Update No. 2016-09 ("ASU 2016-09"), Compensation-Stock Compensation (Topic 718), as part of its simplification initiative. The areas for simplification in ASU 2016-09 involve several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. ASU 2016-09 is effective for periods beginning after December 15, 2016 with early adoption permitted. The adoption of this standard on January 1, 2017 did not have a material impact on our consolidated financial statements and related disclosures.

In November 2015, the FASB issued Accounting Standards Update No. 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 adoption of this standard on January 1, 2017 did not have a material impact on our consolidated financial statements and related disclosures.

Issued Accounting Pronouncements – Not Yet Adopted

In August 2018, the Securities Exchange Commission ("SEC") adopted the final rule under SEC Release No. 33-10532, Disclosure Update and Simplification, amending certain disclosure requirements that were redundant, duplicative, overlapping, outdated or superseded. In addition, the amendments expanded the disclosure requirements on the analysis of stockholders' equity for interim financial statement. Under the amendments, an analysis of changes in each caption of stockholders' equity presented in the balance sheet must be provided in a note or separate statement. The analysis should present a reconciliation of the beginning balance to the ending balance of each period

for which a statement of comprehensive income is required to be filed. The Company anticipates its first presentation of changes in stockholders' equity as required under the new SEC guidance will be included in its Form 10-Q for the three-month period ended March 31, 2019.

In August 2018, the FASB issued Accounting Standards Update No. 2018-15 ("ASU 2018-15"), Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract. ASU 2018-15 clarifies certain aspects of ASU 2015-05, Customer's Accounting for Fees Paid in a Cloud Computing Arrangement, which was issued in April 2015. Specifically, ASU 2018-15 aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal use software (and hosting arrangements that include an internal-use software license). ASU 2018-15 is effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years with early adoption permitted. The Company is currently evaluating the impact its adoption may have on its consolidated financial statements.

In July 2018, the FASB issued Accounting Standards Update No. 2018-09 ("ASU 2018-09"), Codification Improvements, which clarify, correct errors in, or make minor improvements to a variety of ASC topics. The changes in ASU 2018-09 are not expected to have a significant effect on current accounting practices. Some of the amendments in this update do not require transition guidance and will be effective upon this update. However, many of the updates do have transition guidance with effective dates for periods beginning after December 15, 2018. The Company does not expect this ASU to have a significant impact on its consolidated financial statements.

In June 2018, the FASB issued Accounting Standards Update No. 2018-07, ("ASU 2018-07"), Compensation—Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting. ASU 2018-07 is intended to simplify aspects of share-based compensation issued to non-employees by making the guidance consistent with the accounting for employee share-based compensation. ASU 2018-07 is required to be adopted for annual periods beginning after December 15, 2018, including interim periods within those fiscal years. Early adoption is permitted. The Company is currently evaluating the impact that the adoption of ASU 2018-07 will have on its consolidated financial statements.

In February 2018, the FASB issued Accounting Standards Update No. 2018-02, ("ASU 2018-02"), Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income. ASU 2018-02 allows a reclassification from accumulated other comprehensive income to retained earnings for stranded tax effects resulting from the Tax Cuts and Jobs Act. ASU 2018-07 is required to be adopted for annual periods beginning after December 15, 2018, including interim periods within those fiscal years. Early adoption is permitted. The Company does not expect this ASU to have a significant impact on its consolidated financial statements.

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. Topic 842 was subsequently amended by ASU 2017-13, Revenue and Leases: Amendments to SEC Paragraphs Pursuant to the Staff Announcement at the July 20, 2017 EITF Meeting and Rescission of Prior SEC Staff Announcements and Observer Comments; ASU 2018-01, Land Easement Practical Expedient for Transition to Topic 842; ASU No. 2018-10, Codification Improvements to Topic 842, Leases; ASU No. 2018-11, Targeted Improvements and ASU No. 2018-20, Narrow Scope Improvements for Lessors.

ASU 2016-02 is effective for the Company on January 1, 2019. The Company expects to adopt the new standard on its effective date. A modified retrospective transition approach is required, applying the new standard to all leases existing on the date of initial application. An entity may choose to use either (1) its effective date or (2) the beginning of the earliest comparative period presented in the financial statements as its date of initial application. The Company expects to adopt the new standard on January 1, 2019 and use the effective date as our date of initial application. Consequently, financial information will not be updated and the disclosures required under the new standard will not be provided for dates and periods before January 1, 2019. The new standard also provides a number of optional practical expedients in transition. The Company expects to elect the package of practical expedients, which permits us not to reassess under the new standard our prior conclusions about lease identification, lease classification and initial direct costs. The Company is currently evaluating the effect that the new guidance will

have on its financial statements and related disclosures and currently believe the most significant effects of the adoption relate to (1) the recognition of new ROU asset and lease liabilities on our balance sheet for our real estate operating leases and (2) providing significant new disclosures for our leasing activities.

ASU 2016-02 also provides practical expedients and certain exemptions for an entity's ongoing accounting post implementation. The Company currently expects to elect the short-term lease recognition exemption for all leases that qualify. This means, for those leases that qualify, we will not recognize ROU assets or liabilities, and this includes not recognizing ROU assets or liabilities for existing short-term leases of those assets in transition. We also currently expect to elect the practical expedient to not separate lease and non-lease components for all of our leases.We expect to complete our assessment of the full financial impact of ASC 842 during the first quarter of 2019.

NOTE 4—LOSS PER SHARE

Basic loss per share ("EPS") is calculated by dividing net 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:

	For the year ended December 31,	
	2018	2017
	(in thousands, except share and per share amounts)	
Net loss (numerator)	$ (43,274)	$ (19,161)
Weighted-average shares (denominator)	28,772,384	19,397,506
Basic and diluted net loss per share	$ (1.50)	$ (0.99)

Potentially dilutive securities outstanding as of December 31, 2018 and 2017 relate to stock options outstanding of 3,357,873 and 2,219,000 shares, respectively.

NOTE 5—ACCRUED LIABILITIES

Accrued short-term liabilities are as follows:

	December 31, 2018	December 31, 2017
	(in thousands)	
Accrued milestone payments	$ 1,475	$ 875
Accrued clinical costs	63	212
Accrued compensation and board fees	1,144	810
Accrued rent	44	—
Other	525	119
Total	$ 3,251	$ 2,016

NOTE 6—LICENSE AGREEMENTS AND COMMITMENTS

The Company has entered into two license agreements and certain other agreements with MSK. The license agreements are further described below as the MSK License Agreement and the CD33 License Agreement. These license agreements with MSK grant the Company certain patent rights and intellectual property rights. In consideration of obtaining the patent rights and intellectual property rights, the Company agreed to make certain payments and issue shares of the Company's common stock to MSK. Certain of the payments are contingent milestone and royalty payments, the terms of which are further described below. Amounts disclosed in footnote 5 for

accrued milestone and royalty payments are inclusive of obligations under the MSK License Agreement and CD33 License Agreement, collectively.

MSK License Agreement

On August 20, 2015, we entered into the MSK License Agreement 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 oncology indications. Upon entering into the MSK License Agreement in 2015 and in exchange for the licenses, we paid MSK an upfront payment, issued 1,428,500 shares of our common stock to MSK and agreed to provide certain anti-dilution rights to MSK. 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. We also recorded expense related to common stock issued related to certain anti-dilution rights held by MSK. See further description in note 7, Stockholders' Equity.

The MSK License Agreement 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 obligated 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 Agreement. Total expensed minimum royalty payments in 2016 under the MSK License Agreement were $1,200,000, upon determination that the payment of such minimum royalties was probable and the amount was estimable in 2016. The accrued minimum royalties were recorded as long-term accrued liabilities as of December 31, 2018 and 2017. We are also obligated to pay MSK certain clinical, regulatory and sales-based milestone payments under the MSK License Agreement. 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 Agreement. Total clinical and regulatory milestones potentially due under the MSK License Agreement 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 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 obligated 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 achievement of certain clinical milestones. The Company has not entered into any sublicenses related to the MSK License Agreement. 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. In addition, the Company may terminate the MSK License Agreement with prior written notice to MSK.

No milestones were expensed in the twelve months ended December 31, 2018. The Company expensed $150,000 in milestones in the twelve months ended December 31, 2017. As of both December 31, 2018, and December 31, 2017, $875,000 of accrued milestone obligations were recorded in accrued short-term liabilities and $300,000 was recorded within accrued long-term liabilities. These milestone-related charges were recorded as research and development expense upon determination that payment of these clinical milestone obligations was probable after satisfying the financing requirements described herein.

Research and development is inherently uncertain and as described above, should such research and development fail, the MSK License Agreement is cancelable at the Company's option. The Company also considered the development risk and 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. Given the uncertainty associated with research and development and the Company's ability to cancel the MSK License Agreement, such regulatory-based obligations were determined not to be probable as of December 31, 2018 and 2017, and therefore have not been accrued.

CD33 License Agreement

On November 13, 2017, we entered into an exclusive license agreement for certain MSK rights in connection with certain CD33 antibodies, which we refer to as the CD33 License Agreement. The CD33 License Agreement obligates 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 obligated to pay annual minimum royalties of $40,000 over the royalty term, increasing to $60,000 once a patent within the licensed rights is issued, and commencing on the tenth anniversary of the CD33 License Agreement. These amounts are non-refundable but are creditable against royalty payments otherwise due under the CD33 License Agreement. We are also obligated to pay MSK certain fees under a sponsored research agreement under the CD33 License Agreement. In addition, milestone payments become due upon achievement of the related clinical, regulatory or sales-based milestone defined in the CD33 License Agreement. 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 CD33 License Agreement. Total potential clinical and regulatory milestones potentially due under the CD33 License Agreement are $550,000 and $500,000, respectively. There are also sales-based milestones that become due should the Company achieve certain amounts of sales of licensed products resulting from the CD33 License Agreement with MSK, with total sales-based milestones potentially due of $7,500,000. Failure by the Company to meet certain conditions under the CD33 License Agreement could cause the related license to such licensed product to be canceled and could result in termination of the arrangement with MSK. In addition, the Company may terminate the CD33 License Agreement with prior written notice to MSK. 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 obligated 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 achievement of certain clinical milestones. The Company has not entered into any sublicenses related to the CD33 License Agreement.

In 2017, the total milestone obligations expensed under the CD33 License Agreement with MSK was $550,000, all of which related to clinical milestones. Such clinical milestone obligations become due either based upon the passage of time or achievement of the related milestone activities. None of these clinical milestone obligations were paid in 2017 or 2018, and the total amount of $550,000 was recorded as accrued long-term liabilities as of December 31, 2018 and December 31, 2017. These milestone-related charges were recorded as research and development expense in 2017. Research and development is inherently uncertain and as described above, should such research and development fail, the CD33 License Agreement is cancelable at the Company's option. The Company considered risks as well as each party's termination rights under the CD33 License Agreement when considering whether any regulatory-based milestone payments and minimum royalty payments, certain of which also contain time-based payment requirements, were probable. Given the uncertainty associated with research and development and the Company's ability to cancel the CD33 License Agreement, such obligations were determined not to be probable as of December 31, 2018 and December 31, 2017 and therefore have not been accrued.

MabVax sublicense agreement

On June 27, 2018, we entered into a sublicense agreement with MabVax Therapeutics Holding, Inc ("MabVax") pursuant to which MabVax has sublicensed to the Company certain of MabVax's patent rights and know-how for development and commercialization of products for the prevention or treatment of neuroblastoma by means of administering a bi-valent ganglioside vaccine, granted to MabVax pursuant to an exclusive license agreement between MabVax and MSK. Under the sublicense agreement, the Company has paid a license fee of $700,000 to MabVax and will pay an additional $600,000 at the first anniversary of the sublicense agreement. The initial license fee of $700,000 was expensed and paid upon execution of the agreement and the continuation fee of $600,000 was accrued in 2018. The Company has agreed to become solely responsible for future amounts payable to MSK and to handle other of MabVax' obligations applicable to the licensed indication towards MSK. This includes the obligation to pay development milestones totaling $1,400,000 and mid single-digit royalty payments to MSK. Research and development is inherently uncertain and as described above, should such research and development fail, the MabVax sublicense agreement is cancelable at the Company's option. The Company considered risks as well as each party's termination rights under the MabVax sublicense agreement when considering whether any milestone payments and minimum royalty payments were probable. Given the uncertainty associated with research and development and the Company's ability to cancel the MabVax sublicense agreement, such obligations were determined not to be probable as of December 31, 2018 and therefore have not been accrued.

Other agreements

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 Agreement. During 2018 and 2017, we incurred research and development expenses of $1,192,000 and $1,160,000, respectively, 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 transferring clinical data, databases, regulatory files and other know-how included in the MSK License Agreement to the Company. On October 11, 2017 the MDSA was amended to increase the resources to approximately three full time employees. During 2018 and 2017, we incurred expenses of $396,000 and $357,000, respectively, under the MDSA.

On June 21, 2017, we entered into a master clinical trial agreement, which we refer to as the CTA, with MSK pursuant to which we committed to fund certain clinical trials at MSK. Under the MSK License Agreement, the funding of clinical activities is limited to a five year period. During 2018 and 2017, we incurred research and development expenses of $3,043,000 and $725,000, respectively, under the CTA.

On June 27, 2017, we entered into two separate core facility service agreements, which we refer to as the CFSAs, with MSK pursuant to which we committed to obtaining certain laboratory services from MSK. During 2018 and 2017, we incurred research and development expenses of $325,000 and $195,000, respectively, under the CFSAs.

On November 13, 2017, we entered into a CD33-sponsored research agreement, which we refer to as the CD33-SRA, with MSK pursuant to which we agreed to pay MSK to provide research services over a period of two years related to the intellectual property licensed under the CD33 License Agreement. During the second half of 2017, we incurred research and development expenses of $88,000 under the CD33-SRA. During 2018 and 2017, we incurred research and development expenses of $670,000 and $88,000, respectively, under the CD33-SRA.

Lease Agreements

In January 2018, the Company entered into a lease agreement in connection with its corporate headquarters in New York. The term of the lease is five years from the 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, which are recognized on a straight-line basis.

Additionally, the Company entered a three-year lease agreement for the lease of certain office space in Denmark in February 2018, as amended in November 2018 and February 2019. The lease is payable in monthly installments of approximately $19,000, which are recognized on a straight-line basis. Until the end of March 2018, the Company, has maintained a lease for certain office space in Denmark as further described in footnote 9, Related Party Transactions.

NOTE 7—STOCKHOLDERS' EQUITY

Authorized Stock

As of December 31, 2018, the Company has authorized a total of 105,500,000 shares, 100,000,000 of which are to be common stock, par value $0.0001 per stock, and 5,500,000 of which are to preferred stock, par value $0.0001 per share.

As of December 31, 2017, the Company has authorized a total of 55,500,000 shares, 50,000,000 of which are to be common stock, par value $0.0001 per stock, and 5,500,000 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 34,193,666 shares of its common stock as of December 31, 2018 and 26,749,666 shares of its common stock as of December 31, 2017.

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, 2018 or December 31, 2017.

Issuances of common stock for MSK License Agreement

In connection with the MSK License Agreement, in August 2015 we issued to MSK 1,428,500 shares of our common stock. 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. In 2016, our aggregate equity financing reached $25,000,000 since inception, and we issued to MSK an additional 999,929 shares of our common stock in order to maintain MSK's 12.5% ownership of the Company. The additional shares were issued at the estimated fair market value of such shares at the time of issuance of $4.38 per share, and the total value of $4,380,000 was charged to expense in the period 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 non-employees

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 physicians who were involved in the development of technology licensed from MSK in consideration for their prior service. These two physicians were employees of MSK on the date of grant. 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 each of 2016 and 2017. In 2018 a total of 448,000 shares were issued to the two researchers, and upon completion of the IPO we issued an additional 96,000 shares such that one of the two grants was fully issued. The issuance was made pursuant to a stock grant agreement and did not result in proceeds to the Company. A total of 400,000 shares are to be issued in each of 2019 and 2020 to one non-employee physician, subject to certain conditions, such that the total grant will have been issued. The total award was expensed at its estimated fair value in 2015, as no future service was required to continue to vest in and receive the shares of common stock. In August 2016, the Company repurchased and retired a total of 83,600 shares from the two non-employees of the Company at an amount equal to the estimated fair value of $4.38 per share. The transaction reduced the Company's shareholders' equity by $366,000.

In April 2018, the Company granted 72,373 common stock options to a non-employee physician employed by MSK under our 2015 Equity Incentive Plan (the 2015 Plan). The options become exercisable over a four-year period, with the first twenty-five percent (25%) exercisable twelve (months) from the date of grant and the remainder becoming exercisable ratably each month over the three years thereafter. The contractual term of the option award is 10 years from the date of grant. The total award was expensed at its estimated fair value in April 2018, as no future service was required by the non-employee to continue to vest in the option grant. The shares will become immediately exercisable upon the occurrence of a change in control, as defined in the 2015 Plan as further described in footnote 8, Stock Options.

Issuance of common stock

In September 2018, we completed an initial public offering and issued 6,900,000 shares of Common Stock at a purchase price of $16.00 per share for an aggregate consideration of $99,507,000, net of issuance costs.

In November 2017, we issued 3,208,552 shares of Common Stock at a purchase price of $9.35 per share for an aggregate consideration of $28,887,000, net of issuance costs.

In October 2017, we issued 5,347,568 shares of Common Stock at a purchase price of $9.35 per share for an aggregate consideration of $49,812,000, net of issuance costs.

In January and February 2017, we issued 1,192,662 shares of Common Stock at a purchase price of $8.50 per share for an aggregate consideration of $10,137,000, net of issuance costs.

NOTE 8—STOCK OPTIONS

2015 Equity Incentive 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 (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 Plan. In addition, the number of shares available for issuance under the 2015 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. The exercise price of options granted under the plans 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 Plan vest according to the schedule specified in the grant agreements, which is generally over a four year period and generally become immediately exercisable upon the occurrence of a change in control, as defined. Upon the adoption of the 2018 Equity Incentive Plan in September 2018, no further grants are allowed under the 2015 Equity Incentive Plan.

2018 Equity Incentive Plan

Our board of directors and stockholders approved and adopted the 2018 Plan, which became effective upon the Company's initial public offering in September 2018 and 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 5,500,000 shares of our common stock, inclusive of the awards previously granted under the 2015 Equity Incentive Plan, are reserved for issuance pursuant to the 2018 Plan. In addition, the number of shares available for issuance under the 2018 Plan will also include an annual increase on the first day of each fiscal year beginning in 2019, equal to 4% of the outstanding shares of common stock as of the last day of our immediately preceding fiscal year. The exercise price of options granted under the plans 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 2018 Plan vest according to the schedule specified in the grant agreements, which is generally over a four year period and generally become immediately exercisable upon the occurrence of a change in control, as defined.

Stock Option Valuation

During the years ended December 31, 2018 and 2017, stock-based compensation expenses for stock option grants were $1,381,000 and $609,000, respectively, for options granted to employees. During 2018 the expenses were recorded as $276,000 in research and development expense and $1,105,000 in general and administrative expense. During 2017 the expenses were recorded as $167,000 in research and development expense and $442,000 in general and administrative expense. During the year ended December 31, 2018, stock-based compensation for stock option grants was $585,000 in research and development expense for options granted to non-employees. The Company did not grant any options to non-employees during the year ended December 31, 2017.

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, 2018	Year Ended December 31, 2017
Risk-free interest rate	2.89 %	2.10 %
Expected term (in years)	6.3	7.0
Expected volatility	57.8 %	58.9 %
Expected dividend yield	— %	— %

The assumptions that the Company used to determine the fair value of the stock options granted to non-employees were as follows, presented on a weighted average basis:

	Year Ended December 31, 2018	Year Ended December 31, 2017
Risk-free interest rate	3.00 %	— %
Expected term (in years)	10.0	—
Expected volatility	62.7 %	— %
Expected dividend yield	— %	— %

The Company recognizes compensation expense for only the portion of awards that 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, 2017	2,219,000	$ 3.21	$ 13,626	8.00
Granted	1,138,873	$ 16.56		
Outstanding and expected to vest at December 31, 2018	3,357,873	$ 7.74	$ 43,224	7.90
Exercisable at December 31, 2018	1,690,153	$ 3.00	$ 29,303	6.89

The weighted average grant-date fair value of stock options granted during the years ended December 31, 2018 and 2017 was $9.58 and $5.59 per share, respectively.

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, 2018, we had $10,848,000 of unrecognized compensation related to employee stock options that are expected to vest over a period of 2.82 years.

NOTE 9—RELATED PARTY TRANSACTIONS

MSK is a shareholder of the Company and under the MSK License Agreement, the CD33 License Agreement, CTA, CFAs, SRA and MDSA, we have expensed costs in the total amount of $5,761,000 and

$3,730,000 in the years ended December 31, 2018 and 2017, respectively, for milestones, minimum royalties, research and development costs and patent activities. Please refer to footnote 6 for additional details on our various agreements with MSK. As of December 31, 2018 and 2017, we had a total of $4,475,000 and $4,587,000, respectively, 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. The lease terminated in March 2018, when the Company moved to a new third-party lease. In addition, the Company has reimbursed Weco Group for certain administrative expenses. The total expenses, including rent, equaled $44,000 and $88,000 during 2018 and 2017, respectively.

NOTE 10—INCOME TAXES

Domestic and foreign loss before income taxes are as follows:

	For The Year Ended December 31, 2018 (thousands)	For The Year Ended December 31, 2017 (thousands)
United States	$ (42,456)	$ (18,975)
Foreign	(818)	(186)
Total	$ (43,274)	$ (19,161)

The Company provided no current and deferred income taxes on net losses of $(43,274,000) and $(19,161,000) for years ended December 31, 2018 and 2017, respectively.

The Tax Cuts and Jobs Act (TCJA) was enacted on December 22, 2017. The TCJA contains significant changes to corporate taxation, including but not limited to, a reduction in the U.S. federal corporate tax rate from a top marginal rate of 35% to 21%, a one-time mandatory transition tax on accumulated foreign earnings, limitation of the deduction for net operating losses to 80% of annual taxable income while providing that the net operating loss carryovers for years after 2017 will not expire, limitation on the amount of research and development expenses deductible per year beginning in years after 2021 and reduction of the Orphan Drug Credit from 50% to 25% of qualified clinical testing expenditures for years after 2017. The TCJA also made changes to the U.S. federal taxation of foreign earnings and to the timing of recognition of certain revenue and expenses and the deductibility of certain business expenses.

In response to the TCJA, the staff of the Securities and Exchange Commission issued Staff Accounting Bulletin No. 118, ("SAB 118") to address the application of U.S. GAAP in situations when a registrant does not have the necessary information available, prepared or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of the TCJA.

As a result of the TCJA being signed into law, the Company recognized a provisional charge of $4,394,000 in the fourth quarter of 2017 related to the re-measurement of its U.S. deferred tax assets at the lower enacted corporate tax rate. Due to the history of net operating losses, the Company is in a full valuation allowance position. As a result, the additional tax expense due to the TCJA was offset by an equal reduction to the valuation allowance, resulting in no net tax impact from the TCJA to the overall financial condition and results of operations of the Company. The Company was allowed a measurement period of up to one year after the enactment date of the TCJA to finalize the recording of the related tax accounting effects. As of December 31, 2018, the Company had completed its accounting for the effects of the TCJA and no adjustments were recorded to the provisional amount.

The difference between income taxes expected at the U.S. federal statutory income tax rate of 21% and 34% for tax years ended December 31, 2018 and December 31, 2017, respectively, and income taxes provided are set forth below:

	December 31, 2018 (thousands)	December 31, 2017 (thousands)
Taxes on income at U.S. federal statutory rate	$ (9,127)	$ (6,515)
State and local taxes, net of federal tax effects	(5,778)	(2,152)
Effect of rate change	(9)	4,394
Foreign tax rate differential	(8)	22
Valuation allowance	16,376	5,196
Tax credits	(1,431)	(958)
Other	(23)	13
Total	—	—

For the year ended December 31, 2017, as a result of the enactment of the Tax Cuts and Jobs Act, there was no impact to our financial position or results associated with a write-off of deferred tax assets due to the rate change from 34% to 21% and their associated valuation allowance, and a one-time mandatory transition tax.

Significant components of the Company's net deferred tax assets/(liabilities) are as follows:

	December 31, 2018 (thousands)	December 31, 2017 (thousands)
Deferred tax assets/(liabilities):		
Acquired Intangibles	$ 2,682	$ 2,381
Accrued bonus	—	200
Unrealized foreign exchange loss	(163)	187
Accrued royalty	415	414
Stock based compensation	981	300
Net operating loss carryforwards	25,285	10,847
Tax credit carryforwards	2,789	1,358
Other	78	—
Total deferred tax assets/(liabilities)	32,067	15,687
Valuation allowance	(32,067)	(15,687)
Net deferred tax assets/(liabilities)	—	—

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, 2018, and 2017, the Company has determined that there were no uncertain tax positions. The Company's tax returns for years 2017, 2016 and 2015 are open for tax examination by U.S. federal and state, and the Danish tax authorities.

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 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, 2018, the Company had U.S. federal and state and local net operating loss ("NOL") carryforwards of approximately $72,323,000, which are available to reduce future taxable income. The Company also had U.S. federal tax credits of $2,789,000 as of December 31, 2018, 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"). The Company has performed an analysis of its

Section 382 ownership changes through December 31, 2018. Due to the large annual limitation, the Company believes that it is more likely than not that none of the net operating loss carryforwards will expire as a result of the limitation from the ownership change under Section 382. The Company also has Danish NOL carryforwards of $1,154,000, which have an indefinite carryforward period.

NOTE 11—OTHER BENEFITS

The Company has established a retirement program for 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 years ended December 31, 2018 and 2017. 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.

On October 1, 2018, the Company adopted a defined contribution 401(k) savings plan (the 401(k) plan) covering all U.S. employees of the Company. Participants may elect to defer a percentage of their pretax or after-tax compensation to the 401(k) plan, subject to defined limitations. The plan allows for a discretionary match by the Company. The Company made no matching contributions to the plan during the years ended December 31, 2018 and December 31, 2017.

NOTE 12—SUBSEQUENT EVENTS

In February 2019, the Company entered a three year lease for a 4,500 square feet laboratory lease in Nutley, New Jersey. Monthly lease payments are approximately $12,000 per month during the lease term.

NOTE 13—QUARTERLY CONSOLIDATED FINANCIAL DATA (unaudited)

(In thousands, except per share amounts)	2018			
	March 31	June 30	September 30	December 31
Loss from operations	$ (7,479)	$(10,258)	$ (11,415)	$(14,078)
Net loss	(7,483)	(10,305)	(11,426)	(14,060)
Net loss per share - basic and diluted	$ (0.28)	$ (0.38)	$ (0.42)	$ (0.42)

	2017			
	March 31	June 30	September 30	December 31
Loss from operations	$ (2,897)	$ (3,230)	$ (3,842)	$ (9,275)
Net loss	(2,890)	(3,191)	(3,827)	(9,253)
Net loss per share - basic and diluted	$ (0.16)	$ (0.18)	$ (0.21)	$ (0.44)

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE.

None.

ITEM 9A. CONTROLS AND PROCEDURES.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated, as of the end of the period covered by this Annual Report, the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act). Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were not effective at the reasonable assurance level as of December 31, 2018, due to material weaknesses in our internal control over financial reporting.

In designing and evaluating our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act), management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives.

Exemption from Management's Report on Internal Control over Financial Reporting for the Fiscal Year Ended December 31, 2018.

This Annual Report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our independent registered public accounting firm due to a transition period established by the rules of the SEC for newly public companies.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting, (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the fourth quarter of the year ended December 31, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Internal Control

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 our initial public 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. 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 years ended December 31, 2017 and 2018, 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. 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

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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 and qualify as a smaller reporting company we expect that:

- we will present 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 as long as we continue to qualify as a smaller reporting company;

- 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 our initial public 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.

ITEM 9B. OTHER INFORMATION.

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item 10 is incorporated by reference to the information set forth in the sections titled “Proposal 1 – Election of Directors,” “Executive Officers of the Company,” and “Information Regarding the Board and Corporate Governance” and “Section 16(a) Beneficial Ownership Reporting Compliance” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 is incorporated by reference to the information set forth in the section titled “Executive Compensation” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS.

The information required by this Item 12 is incorporated by reference to the information set forth in the sections titled “Security Ownership of Certain Beneficial Owners and Management” and “Executive Compensation” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE.

The information required by this Item 13 is incorporated by reference to the information set forth in the sections titled “Transactions with Related Persons” and “Information regarding the Board of Directors and Corporate Governance” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES.

The information required by this Item 14 is incorporated by reference to the information set forth in the section titled “Principal Accountant Fees and Services” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES.

(a)1. Financial Statements:
The financial statements listed in the Index to Financial Statements beginning on page F-1 are filed as part of this Annual Report on Form 10-K.

(a)2. Financial Statement Schedules:
There are no Financial Statement Schedules included with this filing for the reason that they are not applicable or are not required or the required information is included in the Financial Statements or Notes listed in the Index to Financial Statements beginning on page F-1.

(a)3. Exhibits
See the Exhibit Index immediately before the signature page of this Annual Report. The exhibits listed in the Exhibit Index below are filed or incorporated by reference as part of this Annual Report.

ITEM 16. FORM 10-K SUMMARY.

None.

EXHIBIT INDEX

Exhibit No.	
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.3 to the Form S-1 filed August 24, 2018).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.4 to the Form S-1 filed August 24, 2018).
4.1	Specimen stock certificate evidencing the shares of common stock (incorporated by reference to Exhibit 4.1 to the Form S-1/A filed September 7, 2018).
4.2	Registration Rights Agreement, dated as of October 13, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.2 to the Form S-1 filed August 24, 2018).
4.3(a)	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.3(a) to the Form S-1 filed August 24, 2018).
4.3(b)	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.3(b) to the Form S-1 filed August 24, 2018).
4.3(c)	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.3(c) to the Form S-1 filed August 24, 2018).
10.1+	License Agreement, dated as of August 20, 2015, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.1 to the Form S-1 filed August 724, 2018).

10.2+	License Agreement, dated as of November 13, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.2 to the Form S-1 filed August 24, 2018).
10.3+	Sponsored Research Agreement, effective as of November 10, 2015, by and between the Registrant and Memorial Sloan Kettering Cancer Center Registrant (incorporated by reference to Exhibit 10.3 to the Form S-1 filed August 24, 2018).
10.4+	Sponsored Research Agreement, dated November 13, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.4 to the Form S-1 filed August 24, 2018).
10.5+	Investigator-Sponsored Master Clinical Trial Agreement, dated as of June 21, 2017, as amended on October 11, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.5 to the Form S-1 filed August 24, 2018).
10.6+	Master Data Services Agreement, dated as of September 23, 2016, as amended on October 11, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.6 to the Form S-1 filed August 24, 2018).
10.7†	Amended and Restated 2015 Equity Incentive Plan (incorporated by reference to Exhibit 10.7 to the Form S-1 filed August 24, 2018).
10.8†	Form of Notice of Grant and Stock Option Agreement under the Amended and Restated 2015 Equity Incentive Plan (incorporated by reference to Exhibit 10.8 to the Form S-1 filed August 24, 2018).
10.9†	2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.9 to the Form S-1 filed August 24, 2018).
10.10†	Form of Stock Option Grant Notice and Stock Option Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.10 to the Form S-1 filed August 24, 2018).
10.11†	Form of Officers and Directors Indemnification Agreement (incorporated by reference to Exhibit 10.11 to the Form S-1 filed August 24, 2018).
10.12†	Service Agreement, effective as of April 1, 2016 between the Registrant and Thomas Gad (incorporated by reference to Exhibit 10.12 to the Form S-1 filed August 24, 2018).
10.13†	Service Agreement, effective as of March 1, 2016 between the Registrant and Dr. Claus Juan Møller San Pedro, M.D., Ph.D. (incorporated by reference to Exhibit 10.13 to the Form S-1 filed August 24, 2018).
10.14†	Service Agreement, effective as of October 1, 2016 between Y-mAbs Therapeutics A/S and Bo Kruse (incorporated by reference to Exhibit 10.14 to the Form S-1 filed August 24, 2018).
10.15	Lease Agreement dated January 10, 2018, by and between the Registrant and RXR HB Owner LLC (incorporated by reference to Exhibit 10.15 to the Form S-1 filed August 24, 2018).
10.16†	Form of Restricted Stock Award Grant Notice and Restricted Stock Award Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.17 to the Form S-1 filed August 24, 2018).
10.17†	Form of Restricted Stock Unit Award Grant Notice and Restricted Stock Unit Award Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.18 to the Form S-1 filed August 24, 2018).

10.18†	Non-Employee Director Compensation Policy (incorporated by reference to Exhibit 10.19 to the Form S-1 filed August 24, 2018).
10.19†	Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.20 to the Form S-1 filed August 24, 2018).
21.1	Subsidiaries of the Registrant (incorporated by reference to Exhibit 21.1 to the Form S-1 filed August 24, 2018).
31.1	Certification of Chief Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act.
31.2	Certification of Chief Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act.
32.1*	Certification of Chief Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act.
32.2*	Certification of Chief Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act.
101.INS	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.

*****The certifications attached as Exhibits 32.1 and 32.2 that accompany this Annual Report on Form 10-K are deemed furnished and not filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.
†Indicates management contract or compensatory plan.
+Portions of the exhibit have been omitted pursuant to an order granted by the Securities and Exchange Commission for confidential treatment.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Company has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Y-MABS THERAPEUTICS, INC.

Dated: March 22, 2019

/s/ THOMAS GAD
Thomas Gad
Founder, Chairman, President and Head of Business Development

POWER OF ATTORNEY

Each person whose signature appears below constitutes and appoints Thomas Gad, acting individually, as his or her true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, 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 connection therewith, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or any of them, or their or his substitutes, may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Company and in the capacities indicated on the 22nd of March 2019.

/s/ THOMAS GAD Thomas Gad	Founder, Chairman of the Board of Directors, President and Head of Business Development
/s/ CLAUS JUAN MøLLER SAN PEDRO Claus Juan Møller San Pedro, M.D., Ph.D	Chief Executive Officer and Director (Principal Executive Officer)
/s/ BO KRUSE Bo Kruse	Executive Vice President, Chief Financial Officer, Secretary Treasurer and Director (Principal Financial Accounting Officer)
/s/ JOHAN WEDELL-WEDELLSBORG Johan Wedell-Wedellsborg	Director
/s/ GREGORY RASKIN Gregory Raskin, M.D.	Director
/s/ GÉRARD BER Gérard Ber	Director
/s/ ASHUTOSH TYAGI Ashutosh Tyagi	Director
/s/ JAMES I. HEALY James I. Healy	Director
/s/ DAVID N. GILL David N. Gill	Director

EXHIBIT 31.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Claus Juan Møller San Pedro certify that:

1. I have reviewed this Annual Report on Form 10-K of Y-mAbs Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. [paragraph omitted in accordance with Exchange Act Rule 13a-14(a)];

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 22, 2019

/s/ Claus Juan Møller San Pedro
Name: Claus Juan Møller San Pedro
Title: Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Bo Kruse, certify that:

1. I have reviewed this Annual Report on Form 10-K of Y-mAbs Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b. [paragraph omitted in accordance with Exchange Act Rule 13a-14(a)];

    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 22, 2019

/s/ Bo Kruse
Name: Bo Kruse
Title: EVP, Chief Financial Officer
(Principal Financial Officer)

EXHIBIT 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER

Pursuant to 18 U.S.C. § 1350, as created by Section 906 of the Sarbanes-Oxley Act of 2002, the undersigned officer of Y-mAbs Therapeutics, Inc. (the "Company") hereby certifies, to his knowledge, that:

(i) the accompanying Annual Report on Form 10-K of the Company for the fiscal year ended December 31, 2018 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

(ii) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: March 22, 2019

/s/ Claus Juan Møller San Pedro
Name: Claus Juan Møller San Pedro
Title: Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 32.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER

Pursuant to 18 U.S.C. § 1350, as created by Section 906 of the Sarbanes-Oxley Act of 2002, the undersigned officer of Y-mAbs Therapeutics, Inc. (the "Company") hereby certifies, to his knowledge, that:

(i) the accompanying Annual Report on Form 10-K of the Company for the fiscal year ended December 31, 2018 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

(ii) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: March 22, 2019

/s/ Bo Kruse
Name: Bo Kruse
Title: EVP and Chief Financial Officer
(Principal Financial Officer)