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

FORM 10-Q

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

For the quarterly period ended September 30, 2019
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)

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

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

Title of each class:	Trading Symbol	Name of each exchange on which registered:
Common Stock, $0.0001 par value	YMAB	NASDAQ Global Select Market

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 the 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 whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒.

There were 39,728,416 shares of Common Stock ($0.0001 par value) outstanding as of November 1, 2019.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, 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 Biological License Application, or BLA, submission and approval by the U.S. Food and Drug Administration, or FDA, and equivalent foreign regulatory authorities and of the anticipated results;
- our pre-clinical studies and future clinical trials for our other product candidates and our research and development programs, whether conducted by us or by any of our collaborators, including the timing of initiation of these trials, 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 sales force;
- our plans for 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;
- 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;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act; and
- our expectations related to the use of proceeds from our prior initial public offering and our recent follow-on shelf public offering.

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 Quarterly Report on Form 10-Q, 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, joint ventures or investments that we may make.

The forward-looking statements contained in this Quarterly Report on Form 10-Q are made as of the date of this Quarterly Report on Form 10-Q, 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.

TABLE OF CONTENTS

You should read this Quarterly Report and the documents we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from the plans, intentions, and expectations disclosed in the forward-looking statements we may make.

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

Y-MABS THERAPEUTICS, INC.

Consolidated Balance Sheets

(unaudited)

(in thousands, except share data)

	September 30, 2019	December 31, 2018
ASSETS		
CURRENT ASSETS		
Cash and cash equivalents	$ 98,192	$ 147,840
Restricted cash	—	31
Other current assets	1,399	3,661
Total current assets	99,591	151,532
Property and equipment, net	1,689	205
Operating lease right-of-use assets	2,086	—
Other assets	318	187
TOTAL ASSETS	$ 103,684	$ 151,924
LIABILITIES AND STOCKHOLDERS' EQUITY		
LIABILITIES		
Accounts payable	$ 7,628	$ 5,872
Accrued liabilities	5,848	3,251
Operating lease liabilities, current portion	516	—
Total current liabilities	13,992	9,123
Accrued milestone and royalty payments	1,932	2,050
Operating lease liabilities, long-term portion	1,821	—
Other liabilities	—	224
TOTAL LIABILITIES	17,745	11,397
Commitments and contingencies (Note 6)		
STOCKHOLDERS' EQUITY		
Preferred stock, $0.0001 par value, 5,500,000 shares authorized at September 30, 2019 and December 31, 2018; none issued at September 30, 2019 and December 31, 2018	—	—
Common stock, $0.0001 par value, 100,000,000 shares authorized at September 30, 2019 and December 31, 2018; 34,593,666 and 34,193,666 shares issued at September 30, 2019 and December 31, 2018, respectively	3	3
Additional paid in capital	228,532	225,352
Accumulated other comprehensive income	131	7
Accumulated deficit	(142,727)	(84,835)
TOTAL STOCKHOLDERS' EQUITY	85,939	140,527
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 103,684	$ 151,924

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

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Net Loss and Comprehensive Loss

(unaudited)

(In thousands, except share and per share data)

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
OPERATING EXPENSES				
Research and development	$ 19,660	$ 8,731	$ 46,665	$ 23,228
General and administrative	4,699	2,684	12,581	5,924
Total operating expenses	24,359	11,415	59,246	29,152
Loss from operations	(24,359)	(11,415)	(59,246)	(29,152)
OTHER INCOME/(EXPENSES)				
Interest and other income/(expenses)	437	(11)	1,354	(62)
NET LOSS	$ (23,922)	$ (11,426)	$ (57,892)	$ (29,214)
Other comprehensive income				
Foreign currency translation	134	39	124	121
COMPREHENSIVE LOSS	$ (23,788)	$ (11,387)	$ (57,768)	$ (29,093)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.70)	$ (0.42)	$ (1.69)	$ (1.08)
Weighted average common shares outstanding, basic and diluted	34,371,927	27,330,579	34,253,739	26,945,432

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

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Changes in Stockholders' Equity

(unaudited)

(In thousands, except share data)

	Common Stock		Additional	Accumulated Other Comprehensive	Accumulated	Stockholders'
	Shares	Amount	Paid-in Capital	Income/(Loss)	Deficit	Equity
Balance December 31, 2017	26,749,666	$ 3	$ 123,879	$ (169)	$ (41,561)	$ 82,152
Stock-based compensation expense	—	—	172	—	—	172
Foreign currency translation	—	—	—	3	—	3
Net loss	—	—	—	—	(7,483)	(7,483)
Balance March 31, 2018	26,749,666	3	124,051	(166)	(49,044)	74,844
Stock-based compensation expense	—	—	904	—	—	904
Foreign currency translation	—	—	—	78	—	78
Net loss	—	—	—	—	(10,305)	(10,305)
Balance June 30, 2018	26,749,666	3	124,955	(88)	(59,349)	65,521
Issuance of common stock in initial public offering, net of issuance costs	6,900,000	—	100,498	—	—	100,498
Issuance of common stock to nonemployees	544,000	—	—	—	—	—
Stock-based compensation expense	—	—	395	—	—	395
Foreign currency translation	—	—	—	40	—	40
Net loss	—	—	—	—	(11,426)	(11,426)
Balance September 30, 2018	34,193,666	$ 3	$ 225,848	$ (48)	$ (70,775)	$ 155,028

	Common Stock		Additional	Accumulated Other Comprehensive	Accumulated	Stockholders'
	Shares	Amount	Paid-in Capital	(Loss)/Income	Deficit	Equity
Balance December 31, 2018	34,193,666	$ 3	$ 225,352	$ 7	$ (84,835)	$ 140,527
Stock-based compensation expense	—	—	864	—	—	864
Foreign currency translation	—	—	—	56	—	56
Net loss	—	—	—	—	(15,934)	(15,934)
Balance March 31, 2019	34,193,666	3	226,216	63	(100,769)	125,513
Stock-based compensation expense	—	—	971	—	—	971
Foreign currency translation	—	—	—	(66)	—	(66)
Net loss	—	—	—	—	(18,036)	(18,036)
Balance June 30, 2019	34,193,666	3	227,187	(3)	(118,805)	108,382
Issuance of common stock to nonemployees	400,000	—	—	—	—	—
Stock-based compensation expense	—	—	1,345	—	—	1,345
Foreign currency translation	—	—	—	134	—	134
Net loss	—	—	—	—	(23,922)	(23,922)
Balance September 30, 2019	34,593,666	$ 3	$ 228,532	$ 131	$ (142,727)	$ 85,939

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

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Cash Flows

(unaudited)

(In thousands)

	Nine months ended September 30,	
	2019	2018
CASH FLOWS FROM OPERATING ACTIVITIES		
Net loss	$ (57,892)	$ (29,214)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	87	15
Stock-based compensation	3,180	1,471
Foreign currency transactions	123	101
Changes in assets and liabilities:		
Other current assets	2,262	(1,881)
Other assets	(131)	(188)
Accounts payable	1,031	1,700
Accrued liabilities and other	2,422	344
NET CASH USED IN OPERATING ACTIVITIES	(48,918)	(27,652)
CASH FLOWS FROM INVESTING ACTIVITIES		
Purchase of property and equipment	(818)	(177)
NET CASH USED IN INVESTING ACTIVITIES	(818)	(177)
CASH FLOWS FROM FINANCING ACTIVITIES		
Proceeds from issuance of common stock, net of issuance costs	—	101,607
Payment of offering costs for private placement	—	(1,002)
NET CASH PROVIDED BY FINANCING ACTIVITIES	—	100,605
Effect of exchange rates on cash, cash equivalents and restricted cash	57	32
NET INCREASE/(DECREASE) IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH	(49,679)	72,808
Cash, cash equivalents and restricted cash at the beginning of period	147,871	90,515
Cash, cash equivalents and restricted cash at the end of period	$ 98,192	$ 163,323
SUPPLEMENTAL DISCLOSURE OF NON-CASH ACTIVITIES		
Property and equipment purchases in accounts payable	$ 752	$ —
Right-of-use assets obtained in exchange for lease obligations	901	—
Common stock issuance costs in accounts payable	—	517
Deferred offering costs included in other assets and accounts payable and accrued liabilities and other	—	851

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

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

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"), 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, and an option to obtain the rights to certain chimeric antigen receptor T-cell, or CAR-T, technologies, as well as rights to certain next-generation humanized, affinity matured bispecific antibodies.

The Company is headquartered in New York, 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; dependence on key personnel; compliance with government regulations and the need to obtain additional financing. The Company's 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 $142.7 million as of September 30, 2019 and $84.8 million as of December 31, 2018. Through September 30, 2019, the Company has funded its operations through proceeds from sales of shares of its common stock, including its initial public offering ("IPO"), in September 2018. As of September 30, 2019, the Company had cash and cash equivalents of $98.2 million, and as of December 31, 2018 the Company had cash and cash equivalents of $147.8 million. As of the issuance date of the quarterly financial statements for the three and nine months ended September 30, 2019, the Company expects that its cash and cash equivalents at September 30, 2019 will be sufficient to fund its operating expenses and capital expenditure requirements through at least the next twelve months. In November 2019, the Company completed a follow-on shelf public offering and raised $134.7 million, net of issuance costs. 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 may be required to raise additional capital to fund future operations through the sale of its equity securities, incurring debt, entering into licensing or collaboration agreements with partners, grants or other sources of financing. Sufficient funds may not be available to the Company at all or on attractive terms when needed from equity or debt financing. If the Company is unable to obtain additional financing from these or other sources when needed, or to the extent needed, it may be necessary to significantly reduce its current rate of spending through delaying, scaling back, or suspending certain research and development programs and other operational programs.

The accompanying unaudited 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") for interim financial information, Accounting Standards Codification ("ASC") Topic 270-10 and with the instructions to Form 10-Q. Accordingly, these financial statements do not include all of the information and notes required by GAAP for complete financial statements. The unaudited interim financial statements include all adjustments (consisting only of normal recurring nature) necessary in the judgment of management for a fair statement of the results for the periods presented. All intercompany balances and transactions have been eliminated. The Company has evaluated subsequent events through the date of this filing. Operating results for the three and nine-month periods ended September 30, 2019 are not necessarily indicative of the results that may be expected for the year ended December 31, 2019, any other interim periods, or any future year or period. The December 31, 2018 consolidated balance sheet data was derived from audited financial statements, but does not include all disclosures required by GAAP. You should read these unaudited interim condensed consolidated financial statements in conjunction with the consolidated financial statements and notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Our critical accounting policies are detailed in our Annual Report on Form 10-K for the year ended December 31, 2018. Effective January 1, 2019, the Company adopted 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; ASU No. 2018-07, Compensation—Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting; Accounting Standards Update No. 2018-02, ("ASU 2018-02"), Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income; and Accounting Standards Update No. 2016-02 ("ASU 2016-02"), Leases. Other than the adoption of the new accounting guidance, our critical accounting policies have not changed materially from December 31, 2018.

Operating Leases

As described below, the Company adopted Topic 842 as of January 1, 2019. The Company determines if an arrangement includes a lease at inception. Operating lease right-of-use assets represent the Company's right to use an underlying asset for the lease term and operating lease liabilities represent its obligation to make lease payments arising from the lease. Operating lease right-of-use assets and liabilities are recognized at the lease commencement date based on the present value of lease payments over the lease term. In determining the net present value of lease payments, the Company uses its estimated incremental borrowing rate based on information available at the lease commencement date. Because most of the Company's leases do not provide an implicit rate of return, an incremental borrowing rate is used based on the information available at the commencement date in determining the present value of lease payments on an individual lease basis. The Company's incremental borrowing rate for a lease is the estimated rate of interest it would have to pay on a collateralized basis to borrow an amount equal to the lease payments under similar terms.

The Company's leases may include options to extend or terminate the lease which are included in the lease term when it is reasonably certain that it will exercise any such options. None of the Company's leases contain any residual value guarantees. Lease expense is recognized on a straight-line basis over the expected lease term. Related variable lease costs incurred are not material to the Company.

Topic 842 also provides practical expedients and certain exemptions for an entity's ongoing accounting post implementation. The Company currently elected the short-term lease recognition exemption for all leases that qualify.

This means, for those leases that qualify, we will not recognize right-of-use assets or liabilities, and this includes not recognizing right-of-use assets or liabilities for existing short-term leases of those assets in transition. We also elected the practical expedient to not separate lease and non-lease components for all of our leases. The Company has made an accounting policy election to account for each separate lease component of a contract and its associated non-lease components as a single lease component. See the Lease Agreements section in Note 6 for the related disclosures.

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, and the valuation of 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.

Segment Information

The Company is engaged solely in the discovery and development of novel antibody-based therapeutic products for the treatment of cancer. Accordingly, the Company has determined that it operates in one operating segment.

Recently Issued Accounting Pronouncements - Adopted

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 adoption of this standard on January 1, 2019 did not have a material impact on our consolidated financial statements and related disclosures.

In June 2018, the FASB issued ASU No. 2018-07, Compensation—Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting (“ASU 2018-07”). 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 adoption of this standard on January 1, 2019 did not have a material impact on our consolidated financial statements and related disclosures.

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 adoption of this standard on January 1, 2019 did not have a material impact on our consolidated financial statements and related disclosures.

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.

The Company adopted the new leasing standards using the modified retrospective transition approach as of January 1, 2019, with no restatement of prior periods or cumulative adjustment to retained earnings. 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 used the effective date as our date of initial application. Consequently, financial information was not updated and the disclosures required under the new standard are not provided for dates and periods before January 1, 2019. The new standard also provides a number of optional practical expedients in transition. The Company elected 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.

Upon adoption of the new leasing standards, the Company recognized a lease liability of $1.8 million and a related right-of-use asset of $1.5 million with the difference being due to the elimination of previously reported deferred rent. The Company subsequently entered into two new lease agreements during the three months ended March 31, 2019, and recognized an incremental lease liability and related right-of-use asset of $0.9 million.

Recently Issued Accounting Pronouncements – Not Yet Adopted

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 2018-14 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.

NOTE 4—NET LOSS PER SHARE

Basic net loss per share (“EPS”) is calculated by dividing net income or loss attributable to common stockholders by the weighted average common stock outstanding. Diluted EPS is calculated by adjusting weighted average common shares outstanding for the dilutive effect of common stock options and restricted stock units. 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:

	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
	(in thousands, except per share amounts)			
Net loss (numerator)	$ (23,922)	$ (11,426)	$ (57,892)	$ (29,214)
Weighted-average shares (denominator)	34,372	27,331	34,254	26,945
Basic and diluted net loss per share	$ (0.70)	$ (0.42)	$ (1.69)	$ (1.08)

Potentially dilutive securities excluded from the computation of diluted earnings per share relate to stock options outstanding and unvested restricted shares and RSUs and totaled 4,124,169 shares as of September 30, 2019 and 2,799,373 shares as of September 30, 2018.

NOTE 5—ACCRUED LIABILITIES

Accrued short-term liabilities at September 30, 2019 and December 31, 2018 are as follows:

	September 30, 2019	December 31, 2018
	(in thousands)	
Accrued milestone and royalty payments	$ 268	$ 1,475
Accrued clinical costs	1,079	63
Accrued compensation and board fees	1,765	1,144
Accrued rent	—	44
Accrued manufacturing costs	2,734	—
Other	2	525
Total	$ 5,848	$ 3,251

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 the consolidated balance sheets 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 B7-H3 antibody, as well as affinity matured versions of these certain antibodies and certain single chain variable fragments (Fv) constructs, and their use for immunotherapy, targeting the treatment of neuroblastoma, diffuse intrinsic pontine glioma (DIPG), osteosarcoma 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 expenses related to common stock issued related to certain anti-dilution rights held by MSK.

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. As of September 30, 2019, $18,000 of the accrued minimum royalties were recorded as short-term accrued liabilities, and $1,182,000 were recorded as long-term accrued liabilities. As of December 31, 2018, all accrued minimum royalties were recorded as long-term accrued liabilities. 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 three or nine months ended September 30, 2019 or 2018. We paid $725,000 under this arrangement in the first quarter of 2019. As of September 30, 2019, $150,000 of accrued milestone obligations were recorded in accrued short term liabilities and $300,000 was recorded within long-term liabilities. As of December 31, 2018, $875,000 of accrued milestone obligations were recorded in accrued short term liabilities and $300,000 was recorded within long-term liabilities. These milestone-related charges were recorded as research and development expense in 2016, 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 September 30, 2019 and December 31, 2018 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 bispecific 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.

No milestones were expensed in the three or nine months ended September 30, 2019 or 2018. None of the clinical milestone obligations previously accrued were paid in the three or nine months ended September 30, 2019 or 2018. The total amount accrued in prior periods of $550,000 was recorded as $100,000 of accrued short-term liabilities and $450,000 of accrued long-term liabilities as of September 30, 2019, and $550,000 as accrued long-term liabilities as of December 31, 2018. 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 September 30, 2019 and December 31, 2018 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 an up-front license fee of $700,000 to MabVax and paid an additional $600,000 upon 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 the fourth quarter of 2018 and paid in the third quarter of 2019. 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 September 30, 2019 and 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 related to the intellectual property licensed under the MSK License Agreement. The SRA was amended on September 13, 2019, and will expire five years from the date of the amendment. During the three months ended September 30, 2019 and 2018, we incurred research and development expenses of $306,000 and $297,000, respectively, under the SRA, and in the nine months ended September 30, 2019 and 2018, we incurred expenses of $918,000 and $891,000, respectively, under the SRA.

On March 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 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 1, 2018 the MDSA was amended to increase the resources to approximately five full time employees. During the three months ended September 30, 2019 and 2018, we incurred expenses of $205,000 and $60,000, respectively, under the MDSA, and in the nine months ended September 30, 2019 and 2018, we incurred expenses of $547,000 and $273,000, 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 the three months ended September 30, 2019 and 2018, we incurred expenses of $513,000 and $363,000, respectively, under the CTA, and in the nine months ended September 30, 2019 and 2018, we incurred expenses of $2,647,000 and $2,504,000, respectively, under the CTA.

On June 27, 2017, we entered into two separate core facility service agreements, which we refer to as the CFAs, with MSK pursuant to which we committed to obtaining certain laboratory services from MSK. During the three months ended September 30, 2019 and 2018, we incurred expenses of $186,000 and $28,000, respectively, under the CFAs, and in the nine months ended September 30, 2019 and 2018, we incurred expenses of $674,000 and $223,000, respectively, under the CFAs.

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 three months ended September 30, 2019 and 2018, we incurred expenses of $174,000 and $167,000, respectively, under the CD33 SRA, and in the nine months ended September 30, 2019 and 2018, we incurred expenses of $521,000 and $501,000, respectively, under the CD33 SRA.

Lease Agreements

In February 2019, the Company entered into a lease agreement in connection with its 4,500 square feet laboratory in New Jersey. The term of the lease is three years from the date the Company occupied the premises, with an option to extend for an additional two years which the Company expects to exercise and has included in the determination of the related lease liability. Fixed rent payable under the lease is approximately $144,000 per annum and is payable in equal monthly installments of approximately $12,000.

In January 2018, the Company entered into a lease agreement in connection with its corporate headquarters in New York, New York. The term of the lease is five years from the date the Company occupied 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.

Additionally, the Company entered into 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. Fixed rent payable under the lease is approximately $227,000 per annum and is payable in equal monthly installments of approximately $19,000.

As described above in Note 3, the Company adopted Topic 842 as of January 1, 2019. Prior period amounts have not been adjusted and continue to be reported in accordance with the Company's historical accounting under Topic 840.

Total operating lease costs were $174,000 and $91,000 for the three months ended September 30, 2019 and 2018, respectively, and $502,000 and $181,000 for the nine months ended September 30, 2019 and 2018, respectively.

During the three months ended September 30, 2019, the expenses were recorded as $129,000 in research and development expense and $45,000 in general and administrative expense. During the three months ended September 30, 2018, the expenses were all recorded in general and administrative expense. During the nine months ended September 30, 2019, the expenses were recorded as $367,000 in research and development expense and $135,000 in general and administrative expense. During the nine months ended September 30, 2018, the expenses were all recorded in general and administrative expense.

Cash paid for amounts included in the measurement of lease liabilities for the three and nine months ended September 30, 2019 was $188,000 and $419,000, respectively, and was included in net cash used in operating activities in the Company's Consolidated Statements of Cash Flows.

Maturities of operating lease liabilities at September 30, 2019 were as follows (in thousands):

	Operating Leases at September 30, 2019
Remainder of 2019	$ 186
Years ending December 31,	
2020	749
2021	753
2022	646
2023	539
Thereafter	77
Total lease payments	2,950
Less: Imputed interest	(613)
Total operating lease liabilities at September 30, 2019	$ 2,337

Disclosures related to periods prior to the adoption of Topic 842:

Future minimum lease payments, including imputed interest, under non-cancelable operating leases at December 31, 2018 were as follows (in thousands):

	Contractual Obligations at December 31, 2018
2019	$ 510
2020	616
2021	616
2022	616
2023	462
Thereafter	64
Total lease payments	2,884
Less: Imputed interest	—
Contractual obligations at December 31, 2018	$ 2,884

Operating lease liabilities are based on the net present value of the remaining lease payments over the remaining lease term. In determining the present value of lease payments, the Company uses its estimate of its incremental borrowing rate based on the information available at the lease commencement date. As of September 30, 2019, the weighted average remaining lease term is 4.1 years and the weighted average discount rate used to determine the operating lease liability was 11.0%.

NOTE 7—STOCKHOLDERS' EQUITY

Authorized Stock

As of September 30, 2019 and December 31, 2018, the Company has authorized a total of 105,500,000 shares, 100,000,000 of which are common stock, par value $0.0001 per share, and 5,500,000 of which are 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 had issued 34,593,666 shares of its common stock as of September 30, 2019 and 34,193,666 shares of its common stock as of December 31, 2018.

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 September 30, 2019 or December 31, 2018.

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 researchers who were involved in the development of technology licensed from MSK in consideration for their prior service. These two researchers were employees of MSK. The shares are released according to a vesting schedule. A total of 560,000 shares were issued in 2015, and 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 non-employee researchers, and upon completion of the IPO, we issued an additional 96,000 shares pursuant to a stock grant agreement and the issuance did not result in proceeds to the Company. A total of 400,000 shares were issued in 2019 and 400,000 shares are to be issued in 2020 to one of the non-employee researchers, 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 one of the non-employee researchers 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 Note 8, Share-Based Compensation.

Issuance of common stock

In August 2019, we issued 400,000 shares of our common stock. The issuance was made pursuant to a stock grant agreement and did not result in proceeds to the Company.

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. Upon completion of the IPO, we also issued 96,000 shares of our common stock pursuant to a stock grant agreement and the issuance did not result in proceeds to the Company.

In August 2018, we issued 448,000 shares of our common stock. The issuance was made pursuant to stock grant agreements and did not result in proceeds to the Company.

NOTE 8—SHARE-BASED COMPENSATION

2015 Equity Incentive Plan

Our board of directors and stockholders have approved and adopted the 2015 Plan, which provided 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 were reserved for issuance pursuant to the 2015 Plan. Options granted under the 2015 Plan vest according to the schedule specified in the grant agreements, which is generally a four year period and generally become immediately exercisable upon the occurrence of a change in control, as defined. Upon the 2018 Equity Incentive Plan (the "2018 Plan') becoming effective in September 2018, no further grants are allowed under the 2015 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 a four year period and generally become immediately exercisable upon the occurrence of a change in control, as defined.

Stock Option Valuation

During the three month periods ended September 30, 2019 and 2018, stock based compensation for stock option grants were $1,324,000 and $395,000, respectively, for options granted to employees and directors. During the three months ended September 30, 2019, the expenses were recorded as $263,000 in research and development expense and $1,061,000 in general and administrative expense. During the three months ended September 30, 2018, the expenses were recorded as $67,000 in research and development expense and $328,000 in general and administrative expense.

During the nine month periods ended September 30, 2019 and 2018, stock based compensation for stock option grants were $3,124,000 and $1,471,000, respectively, for options granted to employees, non-employees and directors. During the nine months ended September 30, 2019, the expenses were recorded as $577,000 in research and development expense and $2,547,000 in general and administrative expense. During the nine months ended September 30, 2018, the expenses were recorded as $192,000 in research and development expense and $694,000 in general and

administrative expense for options granted to employees and directors. During the nine months ended September 30, 2018, $585,000 was recorded in research and development expense for options granted to non-employees.

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, 2018	3,357,873	$ 7.74	$ 43,224	7.90
Granted	356,000	$ 21.50		
Outstanding and expected to vest at September 30, 2019	3,713,873	$ 9.06	$ 63,144	7.39
Exercisable at September 30, 2019	2,243,931	$ 4.04	$ 49,408	6.43

There were no stock options granted during the three months ended September 30, 2019. The weighted average fair value of stock options granted during the three months ended September 30, 2018 was $7.54.

The weighted average fair value of stock options granted during the nine month ended September 30, 2019 and 2018 was $12.34 and $6.77, 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 September 30, 2019, we had $12.1 million of unrecognized compensation related to employee stock options that is expected to vest over a period of 2.63 years. As of December 31, 2018, we had $10.8 million of unrecognized compensation related to employee stock options that are expected to vest over a period of 2.82 years.

Restricted Stock Unit Activity

During the three and nine months ended September 30, 2019, stock-based compensation for restricted stock unit grants was $21,000 and $56,000, respectively. During the three months ended September 30, 2019, the expenses were recorded as $18,000 in research and development expense and $2,000 in general and administrative expense. During the nine months ended September 30, 2019, the expenses were recorded as $50,000 in research and development expense and $6,000 in general and administrative expense. There was no stock based compensation for restricted stock units during the three or nine months ended September 30, 2018.

The following table summarizes restricted stock units issued and outstanding:

	Restricted Stock Units
Outstanding and expected to vest at December 31, 2018	—
Granted	10,296
Outstanding and expected to vest at September 30, 2019	10,296

The weighted average fair value of restricted stock units granted during the three and nine months ended September 30, 2019 was $23.11. As of September 30, 2019, we had $183,000 of unrecognized compensation related to employee restricted stock units that are expected to vest over a period of 2.30 years.

NOTE 9—RELATED PARTY TRANSACTIONS

MSK is a shareholder of the Company. Under the MSK License Agreement, the CD33 License Agreement, CTA, CFAs, SRA and MDSA, we have expensed costs in the total amount of $1,384,000 and $914,000 in the three months ended September 30, 2019 and 2018, respectively, for milestones, minimum royalties, and research and development costs. We expensed costs in the total amounts of $5,307,000 and $4,526,000 in the nine months ended September 30, 2019 and 2018, respectively, under these agreements with MSK. Please refer to Note 6 for additional details on our agreements with MSK. As of September 30, 2019 and December 31, 2018, we had a total of $3,829,000 and $4,475,000, respectively, recorded as accounts payable and accrued liabilities related to amounts due to MSK.

NOTE 10—INCOME TAXES

The Company provided no current and deferred income taxes on net losses of $23.9 millon and $11.4 million for the three month periods ended September 30, 2019 and 2018, respectively, and the net losses of $57.9 million and $29.2 million for the nine month periods ended September 30, 2019 and 2018, respectively.

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 September 30, 2019 and December 31, 2018, the Company has determined that there were no uncertain tax positions. The Company's tax returns for years 2018, 2017, 2016, and 2015 are open for tax examination by U.S. federal and state, and the Danish tax authorities.

The valuation allowance related primarily to net U.S. deferred tax assets from operating losses, research and development tax credit carryforwards, and acquired intangibles.

The Company maintains a full valuation allowance on its U.S. and foreign deferred tax assets. The assessment regarding whether a valuation allowance is required considers both positive and negative evidence when determining whether it is more-likely-than-not that deferred tax assets are recoverable. In making this assessment, significant weight is given to evidence that can be objectively verified. In its evaluation, the Company considered its cumulative loss in recent years and its forecasted losses in the near-term as significant negative evidence. Based upon review of available positive and negative evidence, the Company determined that the negative evidence outweighed the positive 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.

NOTE 11—OTHER 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 three or nine months ended September 30, 2019 and 2018.

The Company has established a retirement program for employees of the Company's 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 three and nine months ended September 30, 2019 and 2018. In addition, health insurance benefits for our Danish employees are fully paid for by such employees. Our Danish subsidiary does not incur any costs for these health insurance benefits.

NOTE 12-SUBSEQUENT EVENTS

In November 2019, the Company completed a follow-on shelf public offering of 5,134,750 shares of its common stock, including underwriters overallotment option, at a public offering price of $28 per share for an aggregate consideration of $134.7 million net of issuance costs.

Item 2. 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 Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2018 on file with the SEC. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q, 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 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 initiate submission of a rolling Biologics License Application, or BLA, for naxitamab in November 2019, with a goal of receiving approval by the U.S. Food and Drug Administration, or FDA, in 2020. For omburtamab, we expect the BLA submission, either via a rolling submission or via a single submission, to be completed by the end of the first quarter of 2020 with the 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 (diethylenetriamine pentaacetate) and huB7-H3, a humanized version of omburtamab, each targeting indications with large adult patient populations.

We have initiated a Phase I trial with our huGD2 bispecific antibody, or BsAb, for the treatment of refractory GD2 positive adult and pediatric solid tumors, 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 in April 2015, we have devoted substantially all of our resources to organizing and staffing our company, business planning, identifying potential product candidates, conducting pre-clinical studies of our product candidates and clinical trials of our lead product candidates, raising capital, and acquiring and developing our technology platform among other matters. We do not have any products approved for sale and have not generated any revenues from product sales.

To date, we have financed our operations primarily through private placements of our securities 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 to the public 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 approximately $230.0 million through September 30, 2019 from the sale and issuance of our common stock. In November 2019, we completed a follow-on shelf public offering and issued 5,134,750 shares at a price to the public of $28.00 per share, which included the exercise in full of the underwriters' option to purchase additional shares for gross proceeds of approximately $143.8 million, before deducting underwriting discounts and commissions and estimated offering expenses.

As of September 30, 2019, we had an accumulated deficit of $142.7 million. Our net losses were $23.9 million and $11.4 million for the three months ended September 30, 2019 and 2018, respectively, and $57.9 million and $29.2 million for the nine months ended September 30, 2019 and 2018, respectively. We have incurred significant net operating losses in every year since our inception and expect to continue to incur increasing net operating losses and significant expenses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly as we:

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

We believe that our cash on hand, including the net proceeds from our follow-on shelf public offering in November 2019, will be sufficient to fund our operations and capital expenditures through the fourth quarter of 2022. 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 Contract Research Organizations, or CROs, and Contract Manufacturing Organizations, or CMOs, to carry out our pre-clinical and clinical development activities and we do not yet have a sales organization.

Moreover, pursuant to the MSK License, we have obtained exclusive rights to MSK's rights in our current product candidates. Under the MSK License, we have committed to funding scientific research and conducting certain clinical trial activities at MSK through 2020. As these product candidates progress through clinical development, regulatory approval and commercialization, certain milestone payments will come due either as a result of the milestones having been met or the passage of time even if the milestones have not been met. Also, we will owe MSK customary royalties on commercial sales of our approved products, including a fixed minimum royalty starting in 2020 whether or not product sales are ever achieved. In addition, we have committed to obtain certain personnel and laboratory services at MSK under our MDSA, and two separate CFAs. Also, under our CTA 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 note 6 "License Agreements and Commitments."

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. 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 CFAs, the CTA, 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 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 patients and 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 and frequency of patient follow-up;
- the results of our clinical trials and pre-clinical studies;
- the establishment of commercial manufacturing capabilities;
- adequate ongoing availability of raw materials and drug substance for clinical development and any commercial sales;
- the receipt of marketing approvals, including a safety, tolerability and efficacy profile that is satisfactory to the FDA or any non-U.S. regulatory authority;
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the commercialization of approved products.

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

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

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

costs, including stock-based compensation, conduct clinical trials and potentially prepare regulatory filings for naxitamab and omburtamab.

General and Administrative Expenses

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

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

Critical Accounting Policies and Significant Judgments and Estimates

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

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Quarterly Report on Form 10-Q, 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 and restricted share units 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 and restricted share units 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 prior to January 1, 2019, 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. No share-based awards were granted to non-employees after December 31, 2018.

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.

Results of Operations

Comparison of the Three Months Ended September 30, 2019 and 2018

The following table summarizes our results of operations for the three months ended September 30, 2019 and 2018:

	Three Months Ended September 30,		
	2019	2018	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 19,660	$ 8,731	$ 10,929
General and administrative	4,699	2,684	2,015
Total operating expenses	24,359	11,415	12,944
Loss from operations	(24,359)	(11,415)	(12,944)
Interest and other income (expense)	437	(11)	448
Net loss	$ (23,922)	$ (11,426)	$ (12,496)

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 MSK and consumable costs, which are simultaneously deployed across multiple projects under development. These costs are included in the table below.

	Three Months Ended September 30,	
	2019	2018
	(in thousands)	
Outsourced manufacturing	$ 11,337	$ 4,028
Licensing agreements	—	—
Clinical trials	1,344	523
Outsourced research and supplies	3,995	2,501
Personnel costs	1,512	981
Professional and consulting fees	401	462
Stock based compensation	281	68
Other	790	168
	$ 19,660	$ 8,731

Research and development expenses increased by $11.0 million, from $8.7 million for the three months ended September 30, 2018 to $19.7 million for the three months ended September 30, 2019. This was primarily due to a $7.3 million increase in outsourced manufacturing expenses for our lead product candidates, naxitamab and omburtamab. In addition, expenses for outsourced research and supplies increased by $1.5 million for the three months ended September 30, 2019, due to our increased need for clinical trial support. Employee-related costs, including salary, benefits and non-cash stock-based compensation for personnel related to our research activities, increased by $0.7 million for the three months ended September 30, 2019, due to our expanding workforce.

General and Administrative Expenses

General and administrative expenses increased by $2.0 million, from $2.7 million for the three months ended September 30, 2018 to $4.7 million for the three months ended September 30, 2019. The increase in general and administrative expenses was primarily attributable to a $0.8 million increase in employee related costs, including salary, benefits and non-cash stock-based compensation for personnel related to our business activities. In addition, costs for setting up commercial infrastructure increased by $0.6 million for the three months ended September 30, 2019. The increase in general and administrative expenses overall primarily relates to the infrastructure and administrative costs of becoming a public company.

Interest and Other Income (Expense)

Other income for the three months ended September 30, 2019 was $437,000 as compared to other expenses of $11,000 for the three months ended September 30, 2018. The increase is attributable to an increase in invested cash balances. Our interest income has not been significant due to low interest earned on our cash balances.

Comparison of the Nine Months Ended September 30, 2019 and 2018

The following table summarizes our results of operations for the nine months ended September 30, 2019 and 2018:

	Nine Months Ended September 30,		
	2019	2018	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 46,665	$ 23,228	$ 23,437
General and administrative	12,581	5,924	6,657
Total operating expenses	59,246	29,152	30,094
Loss from operations	(59,246)	(29,152)	(30,094)
Interest and other income (expense)	1,354	(62)	1,416
Net loss	$ (57,892)	$ (29,214)	$ (28,678)

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 MSK and consumable costs, which are simultaneously deployed across multiple projects under development. These costs are included in the table below.

	Nine Months Ended September 30,	
	2019	2018
	(in thousands)	
Outsourced manufacturing	$ 23,273	$ 8,747
Licensing agreements	—	700
Clinical trials	4,415	2,769
Outsourced research and supplies	11,504	6,515
Personnel costs	4,258	2,240
Professional and consulting fees	1,006	939
Stock based compensation	627	777
Other	1,582	541
	$ 46,665	$ 23,228

Research and development expenses increased by $23.5 million, from $23.2 million for the nine months ended September 30, 2018 to $46.7 million for the nine months ended September 30, 2019. This was primarily due to a $14.5 million increase in outsourced manufacturing expenses for our lead product candidates, naxitamab and omburtamab. In addition, expenses for outsourced research and supplies increased by $5.0 million, from $6.5 million for the nine months ended September 30, 2018 to $11.5 million for the nine months ended September 30, 2019, due to our increased need for clinical trial support. Employee-related costs including salary, benefits and non-cash stock-based compensation for personnel related to our research activities, increased by $1.9 million for the nine months ended September 30, 2019, due to our expanding workforce.

General and Administrative Expenses

General and administrative expenses increased by $6.7 million, from $5.9 million for the nine months ended September 30, 2018 to $12.6 million for the nine months ended September 30, 2019. The increase in general and administrative expenses was primarily attributable to a $3.6 million increase in employee related costs, including salary, benefits and non-cash stock-based compensation for personnel related to our business activities. In addition, costs for setting up commercial infrastructure increased by $1.4 million for the nine months ended September 30, 2019. The increase in general and administrative expenses overall primarily relates to the infrastructure and administrative costs of becoming a public company.

Interest and Other Income/(Expense)

Other income for the nine months ended September 30, 2019 was $1,354,000 as compared to other expenses of $62,000 for the nine months ended September 30, 2018. The increase is attributable to an increase in invested cash balances. Our interest income has not been significant due to low interest earned on our 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 September 30, 2019 primarily through gross proceeds of approximately $230.0 million from the sale of our common stock, including the completion of our IPO on September 25, 2018, in which we issued and sold 6,900,000 shares of our common stock at a public offering price of $16.00 per share, resulting in gross proceeds of approximately $110.4 million before deducting underwriters discounts, commissions and estimated offering costs. In November 2019, we completed a follow-on shelf public offering and issued 5,134,750 shares at a price to the public of $28.00 per share, which included the exercise in full of the underwriters' option to purchase additional shares for gross proceeds of approximately $143.8 million, before deducting underwriting discounts and commissions and estimated offering expenses.

As of September 30, 2019, we had cash and cash equivalents of approximately $98.2 million. Adding the net proceeds of $134.7 million from the follow-on shelf public offering completed in November 2019, we believe that our cash on hand will be sufficient to fund our operations and capital expenditures through the fourth quarter of 2022.

The Company may be required to raise additional capital to fund future operations through the sale of its equity securities, incurring debt, entering into licensing or collaboration agreements with partners, grants or other sources of financing. Sufficient funds may not be available to the Company at all or on attractive terms when needed from equity or debt financing. If the Company is unable to obtain additional financing from these or other sources when needed, or to the extent needed, it may be necessary to significantly reduce its current rate of spending through delaying, scaling back, or suspending certain research and development programs and other operational goals.

Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2019 and 2018:

	Nine Months Ended September 30,	
	2019	2018
Cash used in operating activities	$ (48,918)	$ (27,652)
Cash used in investing activities	(818)	(177)
Cash provided by financing activities	—	100,605
Effect of exchange rates on cash, cash equivalents and restricted cash	57	32
Net increase/(decrease) in cash, cash equivalents and restricted cash	$ (49,679)	$ 72,808

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 $48.9 million for the nine months ended September 30, 2019, as compared to $27.7 million for the nine months ended September 30, 2018. The $21.2 million increase in net cash used in operations was primarily due to an increase of $28.7 million in net loss for the nine months ended September 30, 2019, which was primarily due to operational expenses related to the development of our lead product candidates, naxitamab and omburtamab, and the expansion of our other business activities. This increase was partially offset by an increase in our accounts payables and accruals adjustment of $1.4 million as a result of increased activity leading to higher balances, as well as an increase in non-cash expenses, including stock-based compensation to employees, which increased by $1.8 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $818,000 for the nine months ended September 30, 2019, as compared to $177,000 for the nine months ended September 30, 2018. The $641,000 increase in net cash used in investing activities was primarily caused by investment in laboratory equipment for our newly established laboratory facilities.

Net Cash Provided by Financing Activities

There were no financing activities during the nine months ended September 30, 2019. Net cash provided by financing activities was $100.6 million during the nine months ended September 30, 2018, which related primarily to the completion of our IPO on September 25, 2018, in which we issued and sold 6,900,000 shares of our common stock at a public offering price of $16.00 per share, resulting in gross proceeds of approximately $110.4 million before deducting underwriters discounts, commissions and estimated offering costs.

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 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 September 30, 2019, together with the net proceeds from our shelf follow-on public offering in November 2019, will enable us to fund our operating expenses and capital expenditure requirements through the fourth quarter of 2022. 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. The amount and timing of 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 Priority Review Vouchers, or PRVs;
- proceeds, if any, from future equity or debt financings;
- 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

A summary of our minimum contractual obligations related to our material outstanding contractual commitments is included in Note 6 of our enclosed financial statements.

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. 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 certain contingent payment, such as milestones and royalties, for which the amount, timing and likelihood 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 milestone, 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 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 MSK CD33 License with prior written notice to MSK. No milestones were expensed during the three or nine months ending September 30, 2019 or September 30, 2018.

On June 27, 2018, we entered into a sublicense agreement, 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 GD2-GD3 Vaccine. MSK originally developed the GD2-GD3 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 decided to move forward with the development of the vaccine, we made 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.

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.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of September 30, 2019 and December 31, 2018, we had cash and cash equivalents of $98.2 million and $147.8 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 September 30, 2019, we had cash and cash equivalents denominated in DKK of $0.2 million, 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 4. 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 Quarterly 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 September 30, 2019, due to material weaknesses in our internal control over financial reporting as described below.

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.

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 quarter ended September 30, 2019 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 in our annual report for the year ended December 31, 2019 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.

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. We have not previously been 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 for the year ended December 31, 2019. 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.

PART II - OTHER INFORMATION

Item 1. 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 material legal proceedings.

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 Quarterly Report on Form 10-Q, 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 each year. As of September 30, 2019, we had an accumulated deficit of $142.7 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 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 have 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. The SRA was amended on September 13, 2019, and will expire five years from the date of the amendment. 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 GD2-GD3 Vaccine. In addition to the upfront payment of $700,000 that we have made under the terms of MabVax Sublicense we have also made 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. 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 September 30, 2019, we had $98.2 million in cash and cash equivalents. We believe that our cash and cash equivalents, together with the net proceeds from our follow-on shelf public offering completed in November 2019, will be sufficient to fund our operations and capital expenditures through the fourth quarter of 2022. 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 public company, we are 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 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.

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 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 to our business. If we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed and investors could lose confidence in our reported financial information. Any of the foregoing occurrences, should they come to pass, could negatively impact the public perception of our company, which could have a negative impact on our stock price.

Risks Related to Product Development and Commercialization

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

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

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

- successful and timely completion of our ongoing clinical trials;
- initiation and successful patient enrollment and completion of additional clinical trials on a timely basis;
- safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority for marketing approval;
- timely receipt of marketing 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 commercial sales 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 Investigation New Drug Applications (“IND”) 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 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 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 and 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 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 European Medicines Agency ("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 establish 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 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 that it has 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, CMOs, other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our current and future CROs, CMOs, other contractors and consultants are vulnerable to damage from computer viruses, cyber-attack, malicious intrusion, breakdown or other significant disruption 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, 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 current clinical trial and future product liability insurance coverage that we may obtain, 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 data before we may begin our planned trials and/or may not accept such additional data as adequate to begin our planned trials.

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

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

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

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

We will rely on third parties to manufacture our product candidates for our pre-clinical studies, and in the case of our lead product candidates, our ongoing clinical trials, as well as any additional clinical trials of our other product candidates we may conduct. We also expect to rely on third parties for the manufacturing process of our product candidates, if approved. Our business could be harmed if those third parties fail to provide us with sufficient quantities of product supplies or product candidates, or fail to do so at acceptable quality levels or prices 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 or 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 Biologics License Application, or 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 Diffuse Intrinsic Pontine Glioma ("DIPG") and Desmoplastic Small Round Cell Tumors, ("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 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, 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 Institutional Review Board or 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 FDA may refuse to accept an accelerated approval pathway for naxitamab and omburtamab and may refuse rolling review of our BLAs which could have a material adverse impact on our development and approval process for these product candidates and our other product candidates

We have not previously submitted a BLA to the FDA or comparable foreign authorities for any of our product candidates.

In July 2019, we announced that in a pre-BLA meeting we reached alignment with the FDA on an accelerated approval pathway for naxitamab and our intention to begin submission of a rolling BLA in November 2019. The FDA agreed that we could submit, and the FDA would review, individual portions of our naxitamab BLA submission on a rolling basis, rather than waiting for all portions of the BLA to be completed before submission. We believe that this rolling BLA submission process will provide us with the opportunity for ongoing communications with and feedback from the FDA. During this rolling submission process, however, the FDA may raise issues and pose questions to us that may delay the completion of the BLA submission and thereby potentially also delay the approval process and delay the acceptance of the complete BLA for filing and the ultimate issuance of any Marketing Authorization. We may not be able to provide a satisfactory or a timely response to FDA questions or we may not be able to timely gather the required data to prepare the BLA submissions as we planned. If we are unable to address all questions or concerns the FDA may raise or if we do not have timely access to the data required for the preparations of the BLA, we may not be able to timely file the BLA and ultimately receive a Marketing Authorization for naxitamab. In addition, irrespective of the alignment we reached with the FDA in our pre-BLA meeting, the FDA retains discretion to decide not to review the portions of our BLA for naxitamab we have already submitted until the submission is deemed to be complete and no assurance can be given that we would be able to satisfactorily or timely answer or resolve all the questions and issues the FDA may pose.

We have not yet had a pre-BLA meeting with the FDA for omburtamab. On October 31, 2019, the FDA informed us that upon its review of our omburtamab pre-BLA meeting submission, our pre-BLA meeting, which was originally scheduled for November 2019, had been converted to a general guidance meeting. Our rolling BLA submission for omburtamab will likely not begin until after a new pre-BLA meeting has taken place. Our pre-BLA

meeting could still take place in 2019, or may be rescheduled for the first quarter of 2020. We have not yet reached alignment with the FDA on an accelerated approval pathway for omburtamab or a rolling BLA process. The FDA may not support an accelerated approval pathway or accept a rolling BLA submission for omburtamab. If we do not reach alignment with the FDA on an accelerated approval pathway for omburtamab or a rolling BLA process or if we do not succeed in compiling the information required for the submission of a rolling BLA in a timely manner, we may not be able to meet our goal of completing our omburtamab BLA submission by the end of the first quarter of 2020, which would delay the ultimate receipt of the potential Marketing Authorization.

Should the FDA refuse to accept an accelerated approval pathway for naxitamab or omburtamab or refuse rolling review of our BLAs for either product candidate, this may result in a delay in obtaining required Marketing Authorizations, which could materially adversely affect our ability to generate revenue from commercialization of the particular product candidate, which would likely result in significant harm to our financial position and adversely impact our stock price. This could also adversely affect the development and approval process for our other product candidates.

The European Medicines Agency, or the EMA, or comparable foreign regulatory authorities, may disagree with our regulatory plans, including our plans to seek conditional marketing authorization, 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. To date, no formal withdrawal agreement has been reached between the United Kingdom and the European Union, despite the passage of the date on which it was expected that the United Kingdom's membership in the European Union would terminate. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, Brexit 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 Break-through Designation, or 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 Orphan Drug Designation or Rare Pediatric Disease Designation (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 Orphan Drug Designation, or ODD, to omburtamab for the treatment of NB. In February 2017, the EMA granted orphan medicinal product designation (also referred to herein as “ODD”) to omburtamab for the treatment of NB. In November 2018, the EMA granted ODD for naxitamab for the treatment of 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 40-50% 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.

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.

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, 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 now agree to offer 70 % 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;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

- 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.

Moreover, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, also amended the ACA, effective January 1, 2019, by increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and closing the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” In December 2018, CMS published a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. In addition, CMS has recently published a final rule that would give states greater flexibility, starting in 2020, in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces.

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.

On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the Tax Act, the remaining provisions of the ACA are invalid as well. While the Texas U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA and our business.

Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 (the Right to Try Act) was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act.

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.

The Trump administration released in May 2018 a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. HHS has started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020. This final rule codified a CMS policy change that was effective January 1, 2019.

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.

The collection and processing of personal data—including health data—is governed by the European Union-wide General Data Protection Regulation, or GDPR, which became applicable on May 25, 2018, replacing the current data protection laws of each European Union Member State. GDPR applies to us through the activities of our wholly-owned subsidiary Y-mAbs Therapeutics A/S, and also to any business, regardless of its location, that provides goods or services to residents in the EU which includes our clinical trial activities in European Union Member States. The GDPR imposes more stringent operational requirements for processors and controllers of personal data, including, for example, special protections for "sensitive information" which includes health and genetic information of data subjects residing in the EU, expanded disclosures about how personal information is to be used, limitations on retention of information, increased requirements pertaining to health data and pseudonymised (i.e., key-coded) data, mandatory data breach notification requirements and higher standards for controllers to demonstrate that they have obtained valid consent for certain data processing activities. GDPR grants individuals the opportunity to object to the processing of their personal information, allows them to request deletion of personal information in certain circumstances, and provides the individual with an express right to seek legal remedies in the event the individual believes his or her rights have been violated. Further, the GDPR imposes strict rules on the transfer of personal data out of the European Union to the United States or other regions that have not been deemed to offer "adequate" privacy protections. The GDPR provides that European Union Member States may make their own further laws and regulations in relation to the processing of genetic, biometric or health data, which could result in differences between Member States, limit our ability to use and share personal data or could cause our costs to increase, and harm our business and financial condition. We are also subject to evolving and strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with European Union data protection laws may result in fines (for example, of up to €20,000,000 or up to 4% of the total worldwide annual turnover of the preceding financial year (whichever is higher) under the GDPR) and other administrative penalties, which may be onerous and adversely affect our business, financial condition, results of operations and prospects. As a result of the implementation of the GDPR, we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. There is significant uncertainty related to the manner in which data protection authorities will seek to enforce compliance with GDPR. For example, it is not clear if the authorities will conduct random audits of companies doing business in the EU, or if the authorities will wait for complaints to be filed by individuals who claim their rights have been violated.

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 proprietary rights including 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 not own, or may have to share, the intellectual property rights obtained in corroboration with any other party, or intellectual property rights obtained relating to improvements of in-licensed products or processes.

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. During examination of our as well as in-licensed patent applications third parties may present observations or submit patents, published patent applications or other prior art which may affect the patentability of the claimed inventions. The costs for obtaining patent protection may be increased significantly by the need for appeal proceedings or oral proceedings, and may result in a patent not being issued. We may become involved in opposition, interference, derivation, post grant review, inter partes review, ex-parte re-examination 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, method of manufacture or method of use;
- we may not be able to prevent parallel importation of products into the U.S., EU member states and/or other jurisdictions, which may reduce our profit margin;
- 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 proprietary 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 infringed the intellectual property of any third party, 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 patents and patents in-licensed from MSK relating to our proprietary technologies and our product candidates are expected to expire on various dates from 2021 through 2035, without taking into account any possible patent term adjustments, extensions or supplementary protection. Our earliest patents in-licensed from MSK of relevance for our products 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 the relevant 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 from MSK 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, extensions or supplementary protections. 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.

Similar considerations pertains to patents granted outside of the U.S., for which the validity, enforceability and/or scope of protection may be influenced by changing national and/or international legal principles.

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 affect technology covered by 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. 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.

We have a sublicense agreement with MabVax Therapeutics Holding, Inc., or MabVax, covering our GD2- GD3 Vaccine. MabVax has filed a voluntary petition in bankruptcy. Bankruptcy proceedings are inherently unpredictable. If the MabVax sublicense or our rights thereunder covering our GD2-GD3 Vaccine are adversely affected by the MabVax bankruptcy proceedings, we may not be able to continue developing our GD2-GD3 Vaccine program.

We have entered into a sublicense agreement, or the MabVax Sublicense, with MabVax pursuant to which MabVax granted us a sublicense to all of the exclusive license rights granted to MabVax under its license agreement with MSK relating to our GD2-GD3 Vaccine. On March 21, 2019, MabVax filed a voluntary petition for relief under Chapter 11 of Title 11 of the United States Bankruptcy Code, or the Bankruptcy Code. MabVax has communicated that it continues to manage and operate its business as a debtor in possession pursuant to the Bankruptcy Code. Bankruptcy proceedings are inherently unpredictable and we cannot be certain that our MabVax Sublicense and our rights thereunder

relating to our GD2-GD3 Vaccine will not be adversely affected by the MabVax bankruptcy proceedings. For example, MSK has objected to the continuation of its license to MabVax, which may, in turn, impact our rights under the MabVax Sublicense. Alternately, MabVax may seek to reject its sublicense agreement with us, subject to any protections available under the Bankruptcy Code for rights in licensed intellectual property; and we may not be able to immediately enforce our rights with respect to any breaches by MabVax of its sublicense agreement with us due to the automatic stay in MabVax's bankruptcy. If the MabVax Sublicense or any of the rights granted to us thereunder are adversely affected by the MabVax bankruptcy proceedings, we may not be able to continue developing our GD2-GD3 Vaccine program.

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 Chief Technical Officer; 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 September 30, 2019, our executive officers, directors and our stockholders, which own more than 5% of our outstanding common stock beneficially own shares representing approximately 54% 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 $32.90 per share through November 1, 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 34,593,666 shares of common stock outstanding as of September 30, 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. As of September 30, 2019 holders of approximately 2,950,000 shares of our common stock have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. We have also registered 6,200,000 shares of common stock that we may issue under our equity compensation plans.

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.

In November 2019, we completed a follow-on shelf public offering and issued 5,134,750 shares of common stock. We may issue our shares of common stock or securities convertible into our common stock from time to time in connection with a financing, acquisition, investments or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and cause the trading price of our common stock to decline.

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) will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the DGCL or our amended and restated certificate of incorporation or amended and restated 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 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Equity Securities

On August 20, 2019, we issued 400,000 shares of our common stock under a stock grant agreement. The issuance did not result in proceeds to the Company. We have deemed this issuance to be exempt from registration under the Securities Act in reliance on Section 4(a)(2), as a transaction by an issuer not involving a public offering. No underwriters were involved in the foregoing issuance of securities.

Use of Proceeds of Our Initial Public Offering

On September 20, 2018, the SEC declared effective our registration statement on Form S-1 (File No. 333-226999), as amended (the "Registration Statement"), filed in connection with our initial public offering, or IPO. The IPO closed on September 25, 2018 and 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 900,000 shares of common stock sold pursuant to the underwriters' full exercise of their option to purchase additional shares. We received gross proceeds from the IPO of $110.4 million, before deducting underwriting discounts and commissions of approximately $7.7 million and estimated offering expenses of approximately $2.9 million. The managing underwriters of the offering were Merrill Lynch, Pierce, Fenner & Smith Incorporated and Cowen and Company, LLC. No offering expenses were paid or are payable, directly or indirectly, to our directors or officers, to persons owning 10% or more of any class of our equity securities or to any of our affiliates.

As of March 31, 2019 and June 30, 2019, we had not used any of the proceeds from the IPO. As of September 30, 2019, we had used approximately $1.6 million of the proceeds from the IPO net proceeds primarily to advance the research and development of our product candidates and for working capital and general corporate purposes. We are holding a significant portion of the balance of the net proceeds from the offering in interest-bearing money market fund. There has been no material change in our planned use of the balance of the net proceeds from what was described in our Prospectus that forms a part of our Registration Statement, which was filed with the SEC on September 24, 2018, pursuant to Rule 424(b)(4) under the Securities Act.

Item 3. Defaults on Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is incorporated herein by reference.

3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K (File No. 001-38650) filed with the Securities and Exchange Commission on September 26, 2018)
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K (File No. 001-38650) filed with the Securities and Exchange Commission on September 26, 2018)
10.1	2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1 (File No. 333-226999) filed with the Securities and Exchange Commission on August 24, 2018)
10.2	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 Registrant's Registration Statement on Form S-1 (File No. 333-226999) filed with the Securities and Exchange Commission on August 24, 2018)
10.3	Form of Restricted Stock Award Grant Notice and Restricted Stock Award Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.16 to the Registrant's Registration Statement on Form S-1 (File No. 333-226999) filed with the Securities and Exchange Commission on August 24, 2018)
10.4	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 10.17 to the Registrant's Registration Statement on Form S-1 (File No. 333-226999) filed with the Securities and Exchange Commission on August 24, 2018)
10.5	Non-Employee Director Compensation Policy (incorporated by reference to Exhibit 10.18 to the Registrant's Registration Statement on Form S-1 (File No. 333-226999) filed with the Securities and Exchange Commission on August 24, 2018) 2018
10.6	2018 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.19 to the Registrant's Registration Statement on Form S-1 (File No. 333-226999) filed with the Securities and Exchange Commission on August 24, 2018)
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1+	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2+	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
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

* Filed herewith.

\+ Furnished herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Y-MABS THERAPEUTICS, INC.

Dated: November 13, 2019

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

Dated: November 13, 2019

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

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 quarterly report on Form 10-Q 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.

Dated: November 13, 2019

By: /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 quarterly report on Form 10-Q 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.

Dated: November 13, 2019

By: /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 Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2019 (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: November 13, 2019

By: /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 Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended September 30, 2019 (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: November 13, 2019

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