Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2020.

OR

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

For the transition period from to

Commission file number 001-38650

Y-mAbs Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

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

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

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

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

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

NONE

(Title of class)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.

Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act.

Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days.

Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files).

Yes ☒ No ☐

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

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

Large accelerated filer ☒ Accelerated filer ☐ Non-accelerated filer ☐ Smaller reporting company ☐ Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined by Rule 12b-2 of the Exchange Act).

Yes ☐ No ☒

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 USC. 7262(b)) by the registered public accounting firm that prepared or issued its audit report.

Yes ☒ No ☐

As of June 30, 2020 the aggregate market value of the common stock held by non-affiliates of the registrant, based on the closing price of a share of common stock as reported by the NASDAQ Global Select Market on such date, was approximately $990.1 million. Shares of the registrant's common stock held by each executive officer, director and holder of 5% or more of the outstanding common stock have been excluded in that such persons may be deemed to be affiliates. This calculation does not reflect a determination that certain persons are affiliates of the registrant for any other purpose. The number of outstanding shares of the registrant's common stock as of February 22, 2021 was 43,526,254.

Documents Incorporated by Reference:

Portions of the Registrant's Definitive Proxy Statement relating to the 2021 Annual Meeting of Stockholders, which will be filed with the Securities and Exchange Commission within 120 days after the end of the Registrant's fiscal year ended December 31, 2020 are incorporated by reference into Part III of this Report.

TABLE OF CONTENTS

FORWARD LOOKING STATEMENTS.

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, including statements regarding our business strategy, future operations and results thereof, future financial position, future revenue, projected costs, prospects, current and prospective products, product approvals, research and development costs, current and prospective collaborations, timing and likelihood of success, plans and objectives of management, expected market growth and future results of current and anticipated products, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “contemplate,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in Part I, Item 1A, "Risk Factors" in this Annual Report on Form 10-K. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this Annual Report on Form 10-K may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

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

Unless expressly indicated or the context requires otherwise, the terms ""Y-mAbs,” "company," "we," "us," and "our" in this document refer to Y-mAbs Therapeutics, Inc., a Delaware corporation, and, where appropriate, its subsidiaries.

SUMMARY OF RISK FACTORS

Our business is subject to a number of risks, including risks that may prevent us from achieving our business objectives or may adversely affect our business, financial condition, results of operations, cash flows, and prospects. These risks are discussed more fully below and include, but are not limited to, risks related to:

- our ability to successfully launch and commercialize DANYELZA® (naxitamab-gqgk), referred to as DANYELZA, for the treatment of relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow, in the United States and in any other jurisdictions where we may receive marketing approval in the future;
- the implementation of our business model and our plans to obtain regulatory approval and develop and commercialize our lead product candidate omburtamab and other product candidates, including the potential clinical efficacy, safety and other benefits thereof;
- the rate and degree of market acceptance and clinical utility for DANYELZA or any current or future product candidate for which we may receive marketing approval;
- the timing of our resubmission and potential approval of our Biological License Application, or BLA, for omburtamab;
- our ability and plans in continuing to build out our commercial infrastructure and successfully launching, marketing, and selling DANYELZA, omburtamab and any current or future product candidate for which we may receive marketing approval, including our plans with respect to the focus and activities of our sales force, the nature of our marketing, market access and patient support activities of DANYELZA and related assumptions;
- the pricing, coverage and reimbursement of, and the extent to which patient assistance programs are utilized for DANYELZA, omburtamab or any current or future product candidate for which we may receive marketing approval;
- our ongoing and future clinical trials for DANYELZA and our lead product candidate omburtamab 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 United States Food and Drug Administration, or FDA, and equivalent foreign regulatory authorities and of the anticipated results;
- our ability to manage our business, operations and clinical development plans and timelines could be adversely affected by the effects of health epidemics, including the recent COVID-19 pandemic, on the manufacturing, clinical trial and other business activities performed by us or by third parties with whom we conduct business, including our contract manufacturers, or CMOs, contract research organizations, or CROs, shippers and others;
- our ability to attract, integrate, manage and retain qualified personnel or key employees or our employees may not be able to come to work as a result of COVID-19;
- 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;
- 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 ability to remediate the material weaknesses in our internal control over financial reporting and failure to comply with Section 404(b) of the Sarbanes-Oxley Act;
- 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;
- our ability to continue to maintain and leverage our relationship with Memorial Sloan Kettering Cancer Center, or MSK, including our exclusive rights to the MSK License and current and future technology and our relationship with MSK as a user of DANYELZA and any future products;
- 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;
- adverse effects on our business, financial condition and results of operations from the global COVID-19 pandemic, including the pace of global economic recovery from the pandemic;
- the impact of government laws and regulations;
- our dependence on, and difficulty to find a suitable replacement for, a small number of third party contract manufacturing organizations that we currently use for the complex and difficult manufacture of our product candidates;
- our ability to comply with healthcare laws and regulations in the United States and any foreign countries, including, without limitation, those applying to the marketing and sale of pharmaceutical products;
- our expectations related to the use of proceeds from our prior initial public offering, or IPO, in 2018 and our public offering in 2019; and
- other risks and uncertainties described in the section herein entitled “Risk Factors”.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in the “Risk Factors” section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make.

PART I

ITEM 1. BUSINESS.

We are a commercial-stage biopharmaceutical company focused on the development and commercialization of novel, antibody based therapeutic products for the treatment of cancer. We are leveraging our proprietary antibody platforms and deep expertise in the field of antibodies to develop a broad portfolio of innovative medicines.

On November 25, 2020, DANYELZA® (naxitamab-gqgk) was approved by the United States Food and Drug Administration, or the FDA, for the treatment, in combination with Granulocyte-Macrophage Colony-Stimulating Factor, or GM-CSF, of pediatric patients one year of age and older and adult patients with relapsed or refractory, or R/R, high-risk neuroblastoma, or NB, in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. We are commercializing DANYELZA in the United States and began shipping small quantities of product in February 2021.

DANYELZA is also currently being investigated in three Phase 2 clinical studies for the treatment of patients with first-line NB, third-line NB, and in relapsed osteosarcoma. In addition, we have an ongoing Phase 2 trial at Memorial Sloan Kettering Cancer Center, or MSK, with our GD2-GD3 Vaccine for the treatment of Stage 4 high-risk NB. We believe the GD2-GD3 Vaccine can potentially serve as an add-on treatment to DANYELZA.

We submitted a Biologics License Application, or BLA, to the FDA for radiolabeled 131I-omburtamab for central nervous system, or CNS, leptomeningeal metastases, or LM, from NB in August 2020, and received a Refusal to File letter from the FDA in October 2020. We are in the process of preparing a resubmission of the BLA and we plan to continue to discuss our resubmission plans with the FDA, including a meeting in March 2021 in order to amend the BLA. Assuming a positive outcome of these discussions, we expect to resubmit our BLA for omburtamab by the end of the second quarter of 2021 or the third quarter of 2021. The reason for the FDA's decision to issue the Refusal to File letter was that upon preliminary review, the FDA determined that certain parts of the Chemistry, Manufacturing and Control (CMC) Module and the Clinical Module of the BLA required further detail. Among other things, the FDA requested that detailed validation data be included in the CMC Module, that clinical study data and external control data be reanalyzed using a propensity score adjusted analysis for important baseline characteristics, such as prior receipt of irradiation, and that further supportive evidence in the form of direct anti-tumor effect be included in the Clinical Module. We have been working closely with the FDA to resolve these issues and have a meeting with the FDA scheduled for March 26, 2021, to discuss adequacy of the external control reanalysis and supporting data to demonstrate direct anti-tumor effect for a BLA resubmission. Assuming a positive outcome of these discussions, we expect to resubmit our BLA for omburtamab by the end of the second quarter of 2021 or the third quarter of 2021. However, we can provide no assurance that the FDA will agree with our proposal or that we will be successful in resubmitting our BLA for omburtamab in this timeline.

Additionally, we are conducting clinical studies with omburtamab in diffuse intrinsic pontine glioma, or DIPG, and desmoplastic small round cell tumor, or DSRCT. We also have an omburtamab follow-on product candidate, ^{177}Lu-omburtamab-DTPA, in Phase 1 for the treatment of medulloblastoma, and in Phase I in adults targeting B7-H3 positive CNS/LM tumors.

We are advancing a new generation of T cell engaging bispecific antibodies, or BsAbs, that may destroy tumor cells by recruitment of host T cells. Our Y-BiClone format contains two binding arms for the tumor target and two binding arms for T cells. This format was designed to have the small binding affinity necessary to recruit T cells. We have successfully opened an investigational new drug application, or IND, for our Phase 2 trial with nivatrotamab, our GD2 BsAb product candidate, in Small Cell Lung Cancer, or SCLC. In addition a Phase 1/2 trial with nivatrotamab, for the treatment of refractory GD2 positive adult and pediatric solid tumors is ongoing. We are also advancing a CD33 BsAb for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage, which we expect to enter clinical trials in 2021. We are advancing a pipeline of other novel BsAbs through late pre-clinical development. 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.

Based on the SADA technology, we are using our proprietary radioimmunotherapy SADA platform to advance a series of antibody constructs, where bispecific antibody fragments bind to the tumor before a radioactive payload is injected in a two-step approach. We also refer to the SADA technology as Liquid Radiation™. We have designated GD2-SADA for potential use in GD2 positive solid tumors, B7-H3-SADA for potential use in prostate cancer, GPA33-SADA for potential use in colon cancer, and HER2-SADA for potential use in breast cancer as our first SADA constructs and expect to file an IND for GD2-SADA in 2021. We believe the SADA technology could potentially improve the efficacy of radiolabeled therapeutics in tumors that have not historically demonstrated meaningful responses to radiolabeled agents.

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.

DANYELZA

DANYELZA, our first FDA-approved product is a recombinant humanized immunoglobulin G, subtype 1k, or IgG1κ, monoclonal antibody or mAb that targets ganglioside GD2, which is highly expressed in various neuroectoderm-derived tumors and sarcomas. DANYELZA received regulatory approval by the FDA on November 25,

2020 for the treatment, in combination with GM-CSF, of pediatric patients 1 year of age and older and adult patients with R/R high-risk NB in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy.

DANYELZA is currently being studied in several clinical trials, including a pivotal-stage multicenter trial (Study 201) which is also designed to satisfy the confirmatory study and post-marketing requirements by the FDA, a Phase 2 clinical trial (Study 16-1643) in front-line NB, a pilot study (Study 17-251) of chemoimmunotherapy for high-risk NB and a Phase 2 clinical trial (Study 15-096) for relapsed osteosarcoma.

We believe DANYELZA has multiple potential advantages over other GD2-targeting antibody-based therapies. In particular, its toxicity profile allows for doses two-and-a-half times greater than existing GD2-targeting antibody-based therapies. Unlike currently approved GD2-targeting therapies for NB, which require 10 to 20 hours of infusion and hospitalization for several days, DANYELZA is administered in approximately 30 to 60 minutes in an outpatient setting. We believe this significantly shorter administration time is an important advantage considering the overall pain associated with treatment.

DANYELZA has been administered to more than 400 patients to date, which formed the safety portion of our BLA submission. Other than DANYELZA, there are no FDA-approved therapies for primary refractory or second-line pediatric NB patients. DANYELZA has also received orphan drug designation, or ODD, and rare pediatric disease designation, or RPDD, from the FDA for the treatment of NB. In addition, on August 20, 2018, DANYELZA received breakthrough therapy designation, or BTD, in combination with GM-CSF, for the treatment of high-risk NB refractory to initial therapy or with incomplete response to salvage therapy in patients greater than 12 months of age with persistent, refractory disease limited to bone marrow with or without evidence of concurrent bone involvement. Finally, in November 2018, the European Commission granted orphan medicinal product designation, or OMPD, for naxitamab for the treatment of NB. While our current clinical efforts for DANYELZA are focused on rare pediatric cancers, we believe that we can potentially expand its application to the treatment of adults with cancers that express GD2. We estimate that there were more than 200,000 new adult patients diagnosed with GD2-positive cancers in the United States in 2017.

Omburtamab

Omburtamab, our lead product candidate, is a murine monoclonal antibody that targets B7-H3, an immune checkpoint molecule that is widely expressed in tumor cells of several cancer types. ^{131}I-omburtamab, which is omburtamab radiolabeled with Iodine-131, is currently being studied in several clinical trials including pivotal-stage development Study 101 and Study 03-133 for the treatment of pediatric patients who have CNS/LM from NB. An analysis of 107 patients with pediatric CNS/LM from NB who were treated with ^{131}I-omburtamab in Study 03-133 demonstrated a median overall survival, or OS, of 50.8 months, as compared to historical median OS of approximately six to nine months. ^{131}I-omburtamab has received ODD and RPDD from the FDA for the treatment of NB, and BTD for the treatment of pediatric patients who have CNS/LM from NB. We submitted a BLA to the FDA for ^{131}I-omburtamab for CNS/LM from NB in August 2020 and received a Refusal to File letter from the FDA in October 2020. The reason for the FDA's decision to issue the Refusal to File letter was that upon preliminary review, the FDA determined that certain parts of the Chemistry, Manufacturing and Control (CMC) Module and the Clinical Module of the BLA required further detail. Among other things, the FDA requested that detailed validation data be included in the CMC Module, that clinical study data and external control data be reanalyzed using a propensity score adjusted analysis for important baseline characteristics, such as prior receipt of irradiation, and that further supportive evidence in the form of direct anti-tumor effect be included in the Clinical Module. We have been working closely with the FDA to resolve these issues and have a meeting with the FDA scheduled for March 26, 2021, to discuss adequacy of the external control reanalysis and supporting data to demonstrate direct anti-tumor effect for a BLA resubmission. Assuming a positive outcome of these discussions, we expect to resubmit our BLA for omburtamab by the end of the second quarter of 2021 or the third quarter of 2021. However, we can provide no assurance that the FDA will agree with our proposal or that we will be successful in resubmitting our BLA for omburtamab in this timeline.

^{124}I-omburtamab, which is omburtamab radiolabeled with Iodine-124, is currently being studied for the treatment of Diffuse Intrinsic Pontine Glioma, or DIPG. ^{131}I-omburtamab is currently also being studied for the treatment of Desmoplastic Small Round Cell Tumors, or DSRCT. Both DIPG and DSRCT are rare, and often fatal, cancers. While

our current clinical efforts are focused on rare pediatric cancers, we believe we can potentially expand omburtamab's application to the treatment of CNS/LM resulting from other adult and pediatric solid tumors expressing B7-H3. We estimate that, in the United States and the EU in 2019, there were more than 30,000 new patients diagnosed with cancer that has metastasized to the CNS/LM, of which the vast majority express B7-H3.

On December 31, 2019, we submitted an Investigational New Drug application, or IND, for ^{177}Lu-omburtamab-DTPA, for the treatment of medulloblastoma and we opened the IND for patients in October 2020. We expect to enroll the first patients in this trial before the end of first quarter of 2021. We have also opened a Phase 1 study with ^{177}Lu-omburtamab-DTPA targeting B7-H3 positive CNS/LM tumors in adults. We expect to enroll the first patients in this trial in the first quarter of 2021.

The Y-BiClone Platform

We are advancing a new generation of T cell engaging bispecific antibodies, or BsAbs, that may destroy tumor cells by recruitment of host T cells. The Y-BiClone format contains two binding arms for the tumor target and two binding arms for T cells. This format was designed to have the minimal binding affinity necessary to recruit T cells. We have successfully opened an IND for our Phase 2 trial with nivatrotamab, our GD2 BsAb product candidate, in Small Cell Lung Cancer, or SCLC. In addition a Phase 1/2 trial with nivatrotamab, for the treatment of refractory GD2 positive adult and pediatric solid tumors is ongoing. Our nivatrotamab program thus addresses large patient populations. In pre-clinical studies, nivatrotamab demonstrated the potential for improved tumor-binding, longer serum half-life and significantly greater T-cell mediated killing compared to existing bispecific constructs. In addition, we are advancing a CD33 BsAb for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage, which we expect to enter clinical testing in 2021. We are also advancing a pipeline of other novel BsAbs through late pre-clinical development. 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.

Overview of Active INDs

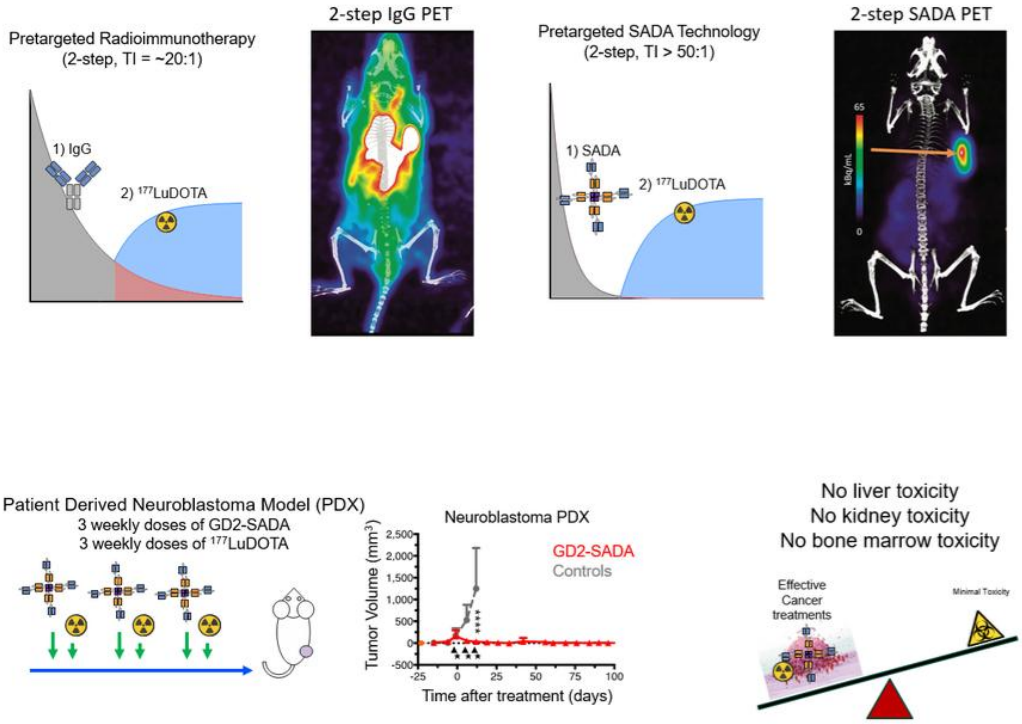
We currently have eight active INDs related to our product candidates. The table below sets forth the product candidate, date of the initial submission of the IND to the FDA, as well as the current sponsor, the subject matter and the current status of each such IND.

Product Candidate	Date of Initial Submission	Current Sponsor	Subject Matter of IND	Current Status
DANYELZA	June 14, 2011	MSK	NB and other GD2 positive tumors	Clinical trials ongoing
Omburtamab (^{131}I-omburtamab and ^{124}I-omburtamab)	September 22, 2000	Y-mAbs (MSK original sponsor)	CNS/LM from NB, DSRCT, DIPG and other B7-H3 positive tumors	Clinical trials ongoing
DANYELZA	September 5, 2017	Y-mAbs	Pediatric NB	Clinical trials ongoing
Nivatrotamab	April 20, 2018	Y-mAbs (MSK original sponsor)	GD2 positive solid tumors	Clinical trial ongoing
Nivatrotamab	December 18, 2020	Y-mAbs	SCLC	Obtained IND in January 2021
^{177}Lu-omburtamab-DTPA	December 31, 2019	Y-mAbs	Medulloblastoma	Obtained IND in October 2020
^{177}Lu-omburtamab-DTPA	September 25, 2020	Y-mAbs	B7-H3 positive CNS/LM tumors in adults patients	Obtained IND in October 2020
GD2-GD3 Vaccine	July 29, 2008	MSK	Pediatric NB	Clinical trial ongoing

The SADA Technology

On April 15, 2020, we entered into a license agreement, or the SADA License Agreement, with MSK and Massachusetts Institute of Technology, or MIT, that grants us an exclusive, worldwide, sublicensable license to certain patent and intellectual property rights developed by MSK and MIT 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 using SADA-BiDE (2-step Self-Assembly and DisAssembly-Bispecific DOTA-Engaging antibody system) Pre-targeted Radioimmunotherapy Platform, or the SADA Technology, a concept we also refer to as Liquid Radiation™. The patents and patent applications covered by the SADA License Agreement are directed, in part, to the SADA Technology, as well as a number of SADA constructs developed by MSK.

We are using the SADA Technology to advance a series of antibody constructs based on the SADA technology, where bispecific antibody fragments bind to the tumor before a radioactive payload is injected in a two-step approach. We also refer to the SADA technology as Liquid Radiation™. We have designated GD2-SADA for potential use in GD2 positive solid tumors, B7-H3-SADA for potential use in prostate cancer, GPA33-SADA for potential use in colon cancer, and HER2-SADA for potential use in breast cancer as our first SADA constructs and expect to file an IND for GD2-SADA in 2021. We believe the SADA technology could potentially improve the efficacy of radiolabeled therapeutics in tumors that have not historically demonstrated meaningful responses to radiolabeled agents.



MSK License Agreements

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

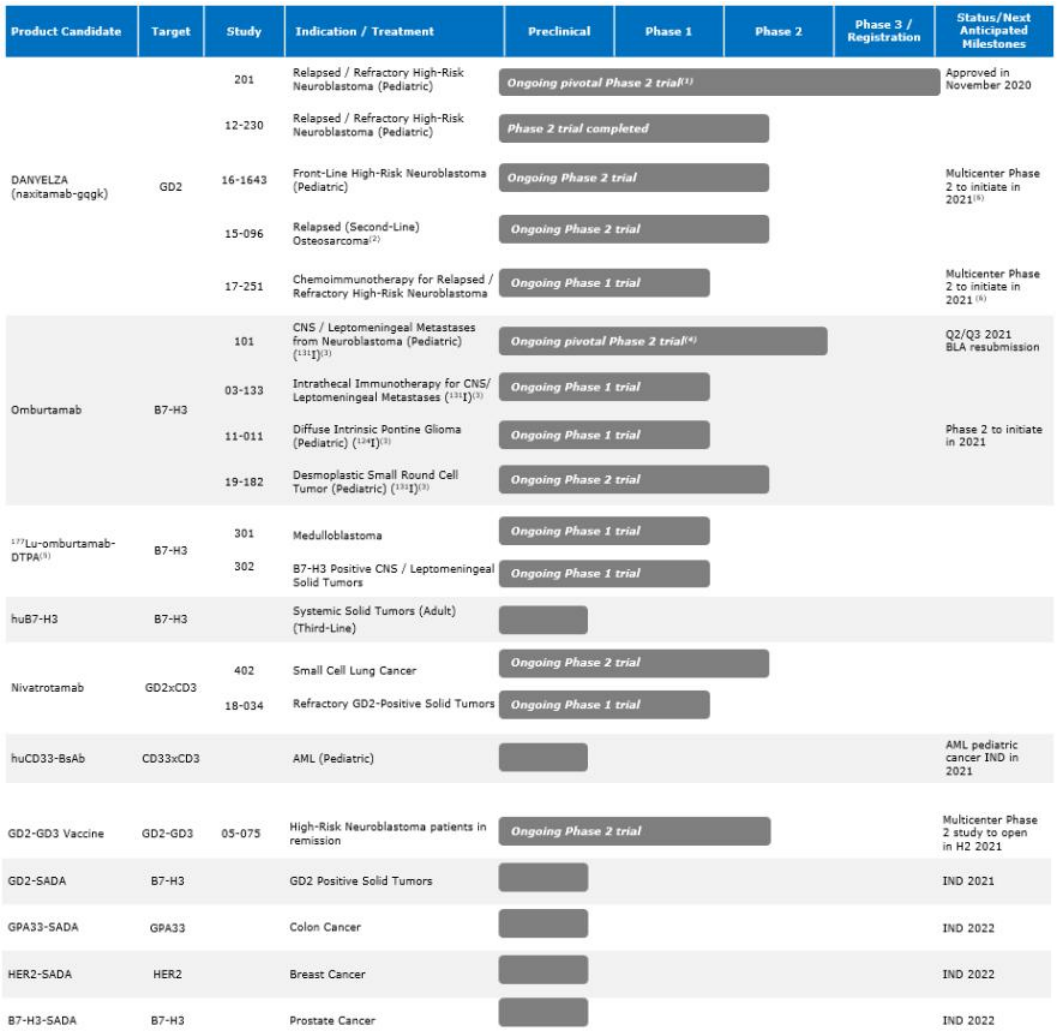
Material Funding Activities

Since our inception in April 2015, we have raised approximately $373.8 million through private placements of our securities, our initial public offering in September 2018 and our public offering in November 2019. As of December 31, 2020, we had cash and cash equivalents of $114.6 million. This number does not include any proceeds

from the sale of our PRV to United Therapeutics, which we received upon FDA approval of DANYELZA and sold for $105 million. Pursuant to the agreement with MSK, we were entitled to retain 60% of the net proceeds from monetization of the PRV, and the remaining 40% was due to MSK. We received our portion of the net proceeds of from the sale of the PRV in the amount of approximately $62.0 million when the transaction was consummated in January 2021. This number does also not include proceeds from our secondary public offering of our common stock pursuant to which we issued and sold 2,804,878 shares of our common stock at a price to the public of $41.00 per share which included the exercise in full of the underwriters' option to purchase additional shares. We received aggregate gross proceeds from our secondary public offering of $115.0 million, when the transaction closed in February 2021.

Our Pipeline

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



(1) DANYELZA was approved by the FDA in November 2020. Pivotal registration studies supporting the BLA submission, comprised of Study 12-230 measuring pharmacokinetic, toxicity and efficacy and an additional pivotal multicenter Phase 2 study, Study 201, designed to prove comparability between study sites using a current good manufacturing practices, or cGMP, commercial manufacturer. Study 201 has also been designed to satisfy the confirmatory study and post-marketing requirements by the FDA.

(2) Initial study represents pediatric and young adult patients.
(3) Represents the radioactive isotope of iodine used to radiolabel omburtamab.
(4) Pivotal registration study designed to support a BLA submission to the FDA, comprised of Study 03-133 measuring pharmacokinetic, toxicity and efficacy and an additional pivotal multicenter Phase 2 study, Study 101, designed to prove comparability between study sites using a cGMP commercial manufacturer. Study 101 has also been designed to satisfy potential confirmatory study and post-marketing requirements by the FDA.
(5) ^{177}Lu-omburtamab-DTPA is a DTPA-conjugated omburtamab labeled with Lutetium-177.
(6) These studies are being planned for potential indication expansion for frontline and in combination with chemotherapy.

Our Business Strategy

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

Key elements of our strategy to achieve this goal are:

- **Independently commercialize DANYELZA in indications and territories where we believe we can maximize the value.** On November 25, 2020, DANYELZA received approval by the FDA following an expedited regulatory pathway and priority review under the BTD granted in 2018. We initiated commercialization of DANYELZA in the United States following the FDA approval. We plan to independently commercialize DANYELZA focusing on already-identified key treatment centers such as MSK, as well as educating doctors, patients and payors about DANYELZA and its current and future indication to drive acceptance and uptake. We believe that we will need to engage a small number of physician specialists for training regarding the appropriate administration and use of DANYELZA. The sales call points for DANYELZA in the United States and the European Union are highly concentrated and generally addressable by a relatively small commercial organization, which we believe will allow us the flexibility to cost-effectively build our own commercial capability. Finally, we have already and we intend to form in the future commercial and development collaborations for indications and in territories that are better served by the resources of larger biopharmaceutical companies.

- **Rapidly and concurrently advance our lead product candidate, omburtamab, to regulatory approval.** We are currently in pivotal stage development for ^{131}I-omburtamab for the treatment of pediatric CNS/LM from NB and we are advancing this lead product candidate through an expedited regulatory pathway and we expect that it will be eligible for priority review under the BTD granted in 2017. We expect to resubmit the BLA for ^{131}I-omburtamab for the treatment of pediatric CNS/LM from NB via a rolling submission by the end of the second quarter of 2021 or the third quarter of 2021. We intend to commercialize omburtamab in the United States as soon as possible after obtaining FDA approval, if such approval occurs.

- **Expand the indications and target patient populations for our existing product candidates.** Our goal is to maximize the potential of our existing product candidates in areas where there is a significant unmet medical need by exploring additional indications, as well as expanding the target population within existing indications. For example, we are developing DANYELZA for the treatment of front-line NB and relapsed osteosarcoma and we intend to discuss our BLA strategy in these indications with the FDA. We are also currently developing radiolabeled omburtamab in clinical trials for the treatment of pediatric patients with DIPG, currently in Phase 1 and DSRCT, currently in Phase 2. After completing our BLA resubmission for ^{131}I-omburtamab for pediatric CNS/LM from NB, we intend to discuss with the FDA the protocol for the continuation and expansion of the ongoing DIPG and DSRCT clinical trials. We believe that we may qualify for a supplemental BLA, or sBLA, in each of these indications assuming positive pivotal data.

- **Advance our novel BsAb product candidates that we believe may offer potential substantial benefits over existing bispecific constructs.** We are also advancing a promising pipeline of BsAbs that we believe have the potential to overcome limitations associated with existing BsAb constructs. Nivatrotamab, our first BsAb product candidate that has entered the clinic is a bivalent humanized anti-GD2 and anti-CD3 BsAb. We are also advancing our CD33-BsAb product candidate for the treatment of hematological

cancers expressing CD33 and expect to file an IND in 2021. Further, we plan to utilize our access to the MULTI-TAG technology platform to create a diverse platform of dimerized BiTEs.

- **Leverage our relationships with leading academic and clinical institutions to develop additional product candidates.** We intend to continue to partner with leading centers, such as MSK, for cancer treatments worldwide, to identify and develop additional product candidates. We believe that our relationship with MSK, our access to several of their technologies and MSK's significant expertise in pediatric cancer care provides us with significant competitive advantages. For example, our Investigator-Sponsored Master Clinical Trial Agreement, or the MCTA, with MSK provides us with ready access to patients for clinical trial enrollment, which is a significant advantage in rare disease drug development where patients are often hard to locate and recruit. Our Sponsored Research Agreement, or the SRA, with MSK, pursuant to which we agreed to provide research funding to MSK, grants us a first option to negotiate an exclusive license to MSK's rights in any new joint inventions discovered under the SRA. We plan to leverage our strong relationship with institutions such as MSK and their expertise and research capabilities to augment our own capabilities in order to identify new product candidates for the treatment of cancers where there is a significant unmet medical need and no effective therapy currently available.

Current Approaches to the Treatment of Cancer

Cancer Overview

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

Cancer is a major public health problem in the United States and worldwide. The American Cancer Society, or ACS, estimated that approximately 40% of all men and women in the United States will be diagnosed with cancer during their lifetime (based on 2011-2013 data). According to the U.S. Centers for Disease Control, cancer is currently the second leading cause of death in the United States and is expected to surpass heart disease as the leading cause of death in the next several years. Although progress has been made in the diagnosis and treatment of cancer, the ACS estimates that an estimated 1.8 million new cancer cases will be diagnosed in the United States and over 600,000 people will have died from cancer in 2020. Thus, there remains a significant need for novel and improved treatment options for cancer patients.

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

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

Cancer therapies are sometimes characterized as front-line, second-line, or third-line, and the FDA often approves new therapies initially only for third-line use. When cancer is detected early enough, front-line therapy is sometimes adequate to effectively treat the cancer or prolong life. Whenever front-line therapy, usually chemotherapy,

radiation therapy, surgery, or a combination of these, proves unsuccessful, second-line therapy may be administered. Second-line therapies often consist of more chemotherapy, surgery, antibody drugs, tumor-targeted therapies such as monoclonal antibodies and small molecule inhibitors, or a combination of these. Third-line therapies can include bone marrow transplantation, antibody and small molecule targeted therapies, more invasive forms of surgery, and new technologies.

Immune System and Introduction to Antibodies

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

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

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

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

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

Our Product and Product Candidates

We have one FDA-approved product and a broad and advanced product pipeline including late-stage and clinically validated product candidates primarily targeting tumors that express GD2 and B7-H3, respectively.

On November 25, 2020, DANYELZA, was approved by the United States Food and Drug Administration, or the FDA, in combination with granulocyte-macrophage colony-stimulating factor, or GM-CSF, for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. Our lead product candidate omburtamab is currently in pivotal stage development for pediatric CNS/LM from NB—a rare

and life-threatening pediatric cancer for which no FDA approved products currently exist. We expect to resubmit the BLA for omburtamab via a rolling submission in the end of the second quarter of 2021 or the third quarter of 2021.

We began to commercialize DANYELZA in the United States upon receipt of FDA approval in November 2020 and we plan to also commercialize omburtamab in the United States as soon as possible after obtaining FDA approval, if such approval occurs. DANYELZA and omburtamab are also in mid-stage clinical development for additional cancers, and we have initiated clinical development for both product candidates in several other indications. Furthermore, we have two omburtamab follow-on product candidates, firstly ^{177}Lu-omburtamab-DTPA for which the INDs for patients with medulloblastoma and adult patients with B7-H3 positive CNS/LM tumors were cleared in October 2020, and secondly, huB7-H3, a humanized version of omburtamab, which is in pre-clinical development. Both product candidates are targeting pediatric oncology as well as larger indications within adult patient populations.

We have successfully opened an IND for our Phase 2 trial with nivatrotamab, our GD2 BsAb product candidate, in Small Cell Lung Cancer. In addition a Phase 1/2 trial with nivatrotamab, for the treatment of refractory GD2 positive adult and pediatric solid tumors is ongoing. Our nivatrotamab program thus addresses large patient populations. In pre-clinical studies, nivatrotamab demonstrated the potential for improved tumor-binding and significantly greater T cell mediated killing compared to existing bispecific constructs. Furthermore, the IgG format exhibits longer halflife as compared to e.g. BITE's hence allowing for weekly or even more sparce dosing. We are also advancing a pipeline of novel BsAbs through late pre-clinical development, including our CD33-BsAb product candidate for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage.

In addition, we have an ongoing Phase 2 trial at MSK, with our GD2-GD3 Vaccine for the treatment of Stage 4 high-risk NB. We believe that the GD2-GD3 Vaccine can potentially serve as an add-on treatment to DANYELZA.

We have exclusive worldwide commercial rights to all of our current product candidates and we have granted commercialization partners certain exclusive rights to develop and commercialize DANYELZA and omburtamab in select jurisdictions, including Greater China, Israel and certain Eastern European countries.

DANYELZA Overview

DANYELZA is a humanized monoclonal antibody approved by the FDA in combination with granulocyte-macrophage colony-stimulating factor, or GM-CSF, for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy, and being evaluated for the treatment of other GD2-positive tumors, including osteosarcoma. DANYELZA targets GD2, which, based on our research, is expressed on almost all of NB cancer cells regardless of disease stage and in almost all osteosarcomas. DANYELZA was granted BTD for treatment of patients with pediatric R/R high-risk NB in 2018.

In clinical studies, DANYELZA has been shown to cause serious infusion reactions including anaphylaxis, cardiac arrest, bronchospasm, stridor, and hypotension. The most common adverse events were mainly mild and moderate and included infusion-related reaction, pain, tachycardia, vomiting, cough, nausea, diarrhea, decreased appetite, hypertension, fatigue, erythema multiforme, peripheral neuropathy, urticaria, pyrexia, headache, edema, anxiety, localized edema and irritability. DANYELZA has been approved with a boxed warning for serious infusion reactions and neurotoxicity.

In November 2018, the European Commission granted orphan medicinal product designation, or OMPD, for naxitamab for the treatment of NB. DANYELZA also received ODD and RPDD from the FDA for the treatment of NB in 2013 and 2017, respectively. The RPDD qualified us for receipt of a PRV upon approval of DANYELZA for treatment of NB, and we did receive such PRV in November 2020. DANYELZA has been administered to more than 400 patients in clinical trials to date.

In pediatric R/R high-risk NB, we believe that DANYELZA has multiple potential advantages over other GD2 targeting antibody-based therapies. In particular, the modest toxicity it exhibits allows for doses 2.5 times greater than

the other GD2 targeting antibody-based therapies. DANYELZA also has a significantly shorter infusion time (approximately 30 to 60 minutes compared to 10 to 20 hours for other GD2 targeting antibody-based therapies being used in front-line therapy), which we believe is important given the pain associated with the therapy and the ability to be administered in an outpatient setting.

In addition, DANYELZA is currently being evaluated in a Phase 2 clinical study (Study 16-1643) in front-line NB, a pilot study (Study 17-251) of chemoimmunotherapy for high-risk NB and a Phase 2 clinical study (Study 15-096) in second-line relapsed osteosarcoma patients.

GD2 Overview

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

As a potential target molecule for anti-tumor therapy, GD2 has certain advantages when compared to other tumor-associated gangliosides because it is highly expressed in tumor cells of several types of cancers and is not expressed at all, or expressed at very low levels, in normal cells. The National Cancer Institute pilot program for the prioritization of the most important cancer antigens ranks GD2 as number 12 out of 75 potential targets for cancer therapy based on therapeutic function, immunogenicity, role of the antigen in oncogenicity, specificity, expression level and percent of antigen-positive cells, stem cell expression, number of patients with antigen-positive cancers, number of antigenic epitopes, and cellular location of antigen expression. GD2 ranks as number six when compared to antigens that are directly targetable on the cell surface. Antibodies directed against GD2 have been shown to effectively induce cell death through a combination of both apoptosis and tumor cell necrosis in GD2-positive tumors.

GD2 Expression in Various Cancer Types

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

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

DANYELZA—mechanism of action

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

DANYELZA for the treatment of pediatric relapsed or refractory high-risk neuroblastoma

On November 25, 2020 DANYELZA® received regulatory approval by the FDA in the United States for treatment in combination with GM-CSF of high-risk R/R NB. This approval was based primarily on interim data from the Study 201 and Study 12-230. In order to meet certain postmarketing commitments issued by the FDA, Study 201 with DANYELZA is currently still ongoing for pediatric R/R high-risk NB. DANYELZA was granted BTD in this indication in 2018. In November 2018, the European Commission granted orphan medicinal product designation, or OMPD, for naxitamab for the treatment of NB. DANYELZA has also received ODD and RPDD from the FDA for the treatment of NB in 2013 and 2017, respectively. The RPDD qualified us for receipt of a PRV upon approval of DANYELZA for treatment of NB by the FDA. The FDA has issued a post-marketing commitment to provide data on PFS, supporting the efficacy of the product. As of February 1, 2021 we have enrolled 54 patients and we anticipate completing the study no later than March 31, 2027. We believe DANYELZA has multiple potential advantages over other GD2 targeting antibodies such as higher doses and administration on an outpatient basis.

In our studies to date, DANYELZA has demonstrated a manageable safety profile, which allows for 2.5 times greater dosing as compared to other GD2 targeting antibody-based therapies. This results in fewer doses per cycle and a significantly shorter infusion time (approximately 30 to 60 minutes versus 10 to 20 hours for dinutuximab). Notably, since severe pain is one of the most common side effects of treatment with GD2 targeting antibody-based therapies, we believe that the ability to reduce infusion time to approximately 30 to 60 minutes is very important for patients and may result in a significant reduction in demand for pain medication such as morphine. These factors allow DANYELZA to be administered in an outpatient setting whereas other GD2 targeting antibody-based therapies require hospitalization which usually lasts for four days or more.

Overview of Neuroblastoma

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

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

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

There are approximately 700 children diagnosed with high-risk NB in the United States each year. We believe the European market is at least one and a half times the size of the U.S. market and that there are approximately 1,050 patients diagnosed with high-risk NB in Europe each year. We believe the current addressable market for DANYELZA consists of approximately 960 new front-line high-risk NB patients each year and 675 primary or second-line eligible R/R NB pediatric patients each year, representing approximately 40% of all pediatric patients diagnosed with NB in the United States and Europe, combined. Moreover, based on the protocol we have developed with MSK, between treatment and maintenance therapy, we believe that typically patients will receive five to ten treatment cycles of DANYELZA, each cycle consisting of three doses.

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

Currently, front-line treatment for pediatric NB patients usually occurs in three stages: induction, consolidation, and maintenance. During the induction phase, patients receive chemotherapy, radiotherapy and possibly surgery to eliminate as much tumor tissue and as many tumor cells as possible. Commonly used agents for induction treatment include cisplatin, etoposide, doxorubicin, cyclophosphamide, and vincristine. Following surgery and/or radiotherapy, most patients enter into consolidation therapy with the goal of eliminating any residual tumor usually with single dose myeloablative agents (e.g. carboplatin-etoposide-melphalan) with stem cell support or an autologous stem cell transplant or repeated transplants with thiotepa-cyclophosphamide followed by cyclophosphamide, etoposide, and ranimustine. Many treatment centers also use immunotherapy as part of the consolidation stage of treatment. Relapse is a frequent occurrence after consolidation.

Other than DANYELZA, there are no approved therapies in the United States for R/R NB patients. Other treatments typically include chemotherapy, radiotherapy and other experimental therapies.

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

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

An earlier murine version of DANYELZA was studied in 17 clinical trials at MSK with a total of more than 800 patients over the last 25 years. DANYELZA has been studied in several clinical trials for the treatment of pediatric R/R NB and other diseases, of which Study 201, Study 15-096, Study 16-1643 and Study 17-251 are currently ongoing. The accelerated approval of DANYELZA by the FDA was based primarily on interim data from Study 201 and Study 12-230.

Study 12-230: Phase 1/2 Study of Combination Therapy of Antibody Naxitamab with Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) in Patients with Relapsed/Refractory High-Risk Neuroblastoma

Phase 1 Portion of Study 12-230

Primary Objective

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

Secondary Objectives

- To study the pharmacokinetics of naxitamab when combined with GM-CSF.
- To assess activity of naxitamab plus GM-CSF against NB.
- To quantitate pain during naxitamab and GM-CSF treatment.
- To study markers of granulocyte-mediated cytotoxicity and NK-mediated cytotoxicity, anti-naxitamab immunity, and anti-tumor immunity before and after treatment with naxitamab/GM-CSF.

- To quantitate the response of NB in BM by quantitative reverse-transcription-polymerase chain reaction, or RT-PCR.

Patient Population

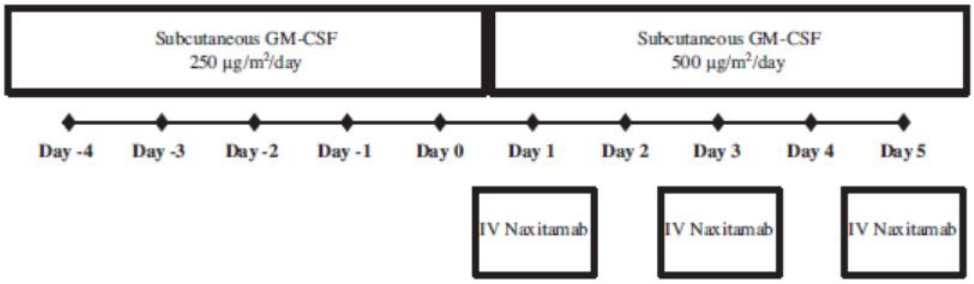
In addition to satisfying certain other criteria, patients must be over one year of age and must have been diagnosed with NB as defined by a) histopathology, or b) BM metastases or Meta-iodobenzylguanidine, or MIBG, avid lesion(s) plus high urine catecholamine levels.

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

Patients will be mainly children and adolescents.

Treatment Protocol

The Phase 1 portion of Study 12-230 assessed dose escalation of intravenous, or IV, naxitamab (days one, three, five) in the presence of subcutaneous GM-CSF (days minus four through five). These three doses of naxitamab and 10 days of GM-CSF constituted a single treatment cycle. Patients who completed 4 cycles without PD were eligible to continue treatment for up to 24 months. For the Phase 2 part of study, patient were eligible to continue treatment for up to 4 cycles after major clinical response was obtained again with a maximum treatment period of 24 months. The diagram below depicts the treatment schedule per cycle in Study 12-230:



Phase 2 Portion of Study 12-230

The Study 12-230 protocol was amended in May 2016 to include an expansion Phase 2 portion. In October 2020, topline results from the first 71 patients (including 29 NED patients) in this Phase 2 study were presented, which continued to show response rates at the same levels as in the dose escalation part of the study with 13 of 15 evaluable, or 87% of, primary refractory patients responding and 7 of 23 evaluable, or 30% of, secondary refractory patients responding.

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

Patient Population

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

Primary Objectives

- In Group 1: (NED patients) To assess the impact of naxitamab and GM-CSF on PFS in patients in greater than or equal to second CR/very good partial response, or VGPR, but at high-risk of another relapse.

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

- In Group 3: To assess the activity of naxitamab and GM-CSF in patients who have secondary refractory disease in the bone and BM by measuring response and by calculating PFS.

Secondary Objectives

- In patients with primary refractory or relapsed disease (groups 1 and 3):

    - To evaluate the PFS from the start of hu3F8+GM-CSF treatment.
    - To evaluate human anti-human antibody (HAHA).

    In patients in >2nd CR (Group 2):

    - To evaluate PFS from the start of hu3F8+GM-CSF treatment.
    - Evaluate event free survival (EFS) from the start of hu3F8+GM-CSF treatment.

- In all patients: To evaluate the safety of naxitamab

Study 12-230 is now closed for recruitment.

Study 201: A Phase 2 Trial of Antibody Naxitamab and Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) in High-Risk Neuroblastoma Patients with Primary or Secondary Refractory Osteomedullary Disease

Study 201 is a single-arm multi-center pivotal study using current Good Manufacturing Practices, or cGMP, manufactured naxitamab, which commenced recruitment in the second quarter of 2018. We have completed the initial enrollment target of 37 patients and continue recruitment at all sites outside the U.S.

Patient population

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

Treatment Protocol

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

Primary Objective

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

Secondary Objectives

- To evaluate the safety of IV naxitamab and GM-CSF.
- To evaluate the duration of response from the start of naxitamab and GM-CSF. Duration of response is defined as the length of time from patient response to PD.
- To evaluate PFS of naxitamab and GM-CSF.
- To evaluate median OS at two years following naxitamab and GM-CSF.
- To evaluate the pharmacokinetics of naxitamab and investigate the formation of HAHAs.

We initiated Study 201 to form the primary basis for our BLA, to establish comparability of study population with Study 12-230 and to satisfy the confirmatory study and post-marketing requirements by the FDA. The FDA granted approval under the accelerated approval regulation. The postmarketing clinical trial required by the FDA to verify and to further characterize the clinical benefit of our ongoing Study 201, which will enroll a minimum of 80 patients and report ORR, DOR, PFS or OS. The ORR is the primary endpoint for the study, DOR is the secondary endpoint, PFS and OS are secondary endpoints in long-term follow up. As of February 1, 2021 we have enrolled 54 patients and we anticipate completing the study no later than March 31, 2027.

Study 16-1643: Naxitamab/GM-CSF Immunotherapy Plus Isotretinoin for Consolidation of First Remission of Patients with High-Risk Neuroblastoma: A Phase 2 Study

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

As of January 2021, 59 patients had completed enrollment in the study which constituted full accrual.

Patient population

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

Patients will mainly be children and adolescents.

Treatment protocol

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

Primary Objective

- To determine two years relapse-free survival.

Secondary Objective

- To determine MRD by using BM specimens.

Safety Results

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

Study 11-009: Phase 1 Study of Naxitamab Monoclonal Antibody in Patients with High-Risk Neuroblastoma and GD2-Positive Tumors

Study 11-009 is a Phase 1 clinical dose escalation study with IV naxitamab given as monotherapy in patients with high-risk NB or other GD2-positive tumors. Safety data from this study was used to support our BLA submission for naxitamab in pediatric R/R high-risk NB. The study closed accrual with 68 patients enrolled. The primary objective of the study is to establish the MTD of naxitamab. The secondary objectives are to study the pharmacokinetics, to assess activity of naxitamab against NB and other GD2-positive tumors, and to quantitate pain during naxitamab treatment.

Two patients experienced reversible DLT of elevated transaminases.

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

Study 17-251 is a ongoing single arm pilot, Phase 2 study at MSK in high-risk R/R NB patients with soft tissue disease. Patients will be treated with naxitamab in combination with irinotecan, temozolomide and sargramostim, or HITS. As of January 2021, 46 patients have been enrolled in the study. If the regimen is found to be acceptable, then we plan to initiate a multicenter Phase 2 study.

Patient population

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

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

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

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

Treatment protocol

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

Primary Objective

- To evaluate the safety of HITS in patients with NB

Secondary Objective

- To evaluate tumor responses to HITS in patients with NB

Safety results

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

DANYELZA for the Treatment of Relapsed Osteosarcoma

DANYELZA is currently being evaluated in an ongoing Phase 2 clinical study (Study 15-096) for the treatment of patients with relapsed osteosarcoma that have been rendered surgically free of evident disease. As of January 2021, 33 patients had been enrolled and we expect to enroll a total of 39 patients. The trial is designed to distinguish between 12-month EFS of 30% versus 50%.

Overview of Osteosarcoma

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

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

DANYELZA for Relapsed Osteosarcoma—Current Treatment Landscape and Associated Limitations

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

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

DANYELZA for Relapsed Osteosarcoma—Clinical Development Program

Currently, DANYELZA is being evaluated in an ongoing Phase 2 clinical trial (Study 15-096) for the treatment of relapsed osteosarcoma. This Phase 2 clinical trial is designed to assess the efficacy of DANYELZA when combined with GM-CSF in patients with relapsed osteosarcoma who have been rendered surgically free of evident disease. The study commenced in July 2015, and as of January 2021, 33 patients had been enrolled. We expect to recruit a total of 39 patients at a total of three US sites. This trial is designed to distinguish between a 12-month EFS of 30% versus 50%.

Study 15-096: A Phase 2 Study of Monoclonal Antibody Naxitamab with Granulocyte-Macrophage Colony Stimulating Factor (GM-CSF) in the Treatment of Recurrent Osteosarcoma

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

Patient Population

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

Treatment Protocol

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

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

Omburtamab Overview

Omburtamab is a novel murine monoclonal antibody currently designed for compartmental immunotherapy, for example in the CNS. Omburtamab targets B7-H3, an immune checkpoint molecule that is widely expressed in tumor cells of several types of cancers. We have radiolabeled omburtamab with either Iodine-131 (^{131}I-omburtamab) or Iodine-124 (^{124}I-omburtamab). ^{131}I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB and was granted BTD in this indication in 2017. In 2016, ^{131}I-omburtamab was granted ODD and RPDD, in each case, for the treatment of NB. The RPDD qualifies us for receipt of a PRV upon approval of omburtamab for treatment of NB, if such approval occurs. An analysis of 107 patients treated through June 2019 demonstrated median OS of 50.8 months (including a five-year median OS of approximately 44%), as compared to historical median OS of approximately six to nine months.

We submitted a BLA to the FDA for ^{131}I-omburtamab for CNS/LM from NB in August 2020 and received a Refusal to File from the FDA in October 2020. The reason for the FDA's decision to issue the Refusal to File letter was that upon preliminary review, the FDA determined that certain parts of the Chemistry, Manufacturing and Control (CMC) Module and the Clinical Module of the BLA required further detail. We are in the process of preparing a

resubmission of the BLA and we plan to continue to discuss our resubmission plans with the FDA, including a meeting in March 2021 in order to amend the BLA. We have been working closely with the FDA to resolve these issues and have a meeting with the FDA scheduled for March 26, 2021, to discuss the adequacy of the external control reanalysis and supporting data to demonstrate direct anti-tumor effect for a BLA resubmission. Assuming a positive outcome of these discussions, we expect to resubmit our BLA for omburtamab by the end of the second quarter of 2021 or the third quarter of 2021. However, we can provide no assurance that the FDA will agree with our proposal or that we will be successful in resubmitting our BLA for omburtamab in this timeline. In addition, radiolabeled omburtamab is in clinical development for two additional rare pediatric cancers, DSRCT, currently in Phase 2 and DIPG, currently in Phase 1. The most recent set of DSRCT data was presented in November 2019. We believe that we are well positioned to submit sBLAs in each of these two indications, assuming positive results in the respective Phase 2 clinical trials, after the potential approval of our BLA for ^{131}I-omburtamab for CNS/LM from NB. Further, we believe that omburtamab has the potential to address CNS/LM metastasis from several other tumors in children and adults that express B7-H3 such as prostate, ovarian, breast, colon, renal, non small cell lung, pancreatic, head and neck cancers, as well as melanoma, glioblastoma, and NB and other small round blue cell tumors of childhood.

B7-H3 Overview

B7-H3 is a member of the B7 family of immune-regulatory ligands. The family includes B7-1, B7-2, PD-L1, PD-L2, B7-H3, B7-H4, B7-H6 and their ligands on T-cells PD-1, CD28, CTLA-4 and ICOS. B7-H3 is highly expressed on many solid cancers and displays high tumor-versus-normal tissue binding differential. In mice, studies have shown that members of the B7 family have the capability to regulate the immune system through both stimulatory and inhibitory signals. Inhibition of certain members of the B7 family has been shown to have significant anti-tumor effects in several solid tumor types. As such, we believe that B7-H3 is a promising target for designing targeted therapeutics with a range of modalities.

B7-H3 Expression in Various Cancer Types

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

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

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

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

consequences. Radiolabeling of omburtamab with either Iodine-124 or Iodine-131 takes place at qualified radiopharmacies according to a well-established procedure.

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

^{131}I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB, and was granted BTD in this indication in 2017. In 2016, ^{131}I-omburtamab was granted ODD and RPDD, in each case, for the treatment of NB. The RPDD qualifies us for receipt of a PRV upon approval of omburtamab for treatment of NB, if such approval occurs. As of June 2019, 107 patients with pediatric CNS/LM from NB had been treated with ^{131}I-omburtamab in Study 03-133. An analysis of these 107 patients demonstrated a median OS of 50.8 months (including an estimated five-year OS of approximately 44%), as compared to historical median OS of approximately six months. ^{131}I-omburtamab can be administered as a push injection in an outpatient setting. We expect to resubmit the BLA for ^{131}I-omburtamab for CNS/LM from NB via a rolling submission by the end of the second quarter of 2021 or the third quarter of 2021. We plan to commercialize ^{131}I-omburtamab in the United States as soon as possible after obtaining FDA approval, if such approval occurs.

Overview of Central Nervous System/Leptomeningeal Metastases from Neuroblastoma

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

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

^{131}I-omburtamab for Pediatric Central Nervous System/Leptomeningeal Metastases from Neuroblastoma—Current Treatment Landscape and Associated Limitations

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

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

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

1. Our review of published research representing 83 patients treated between 1979 and 2013 showed a median OS of 5.6 months (95% CI of three to eight months) for patients with R/R NB who have CNS/LM from NB. We also performed a restricted analysis after removing patients who died before receiving therapy for the CNS/LM from NB disease and only received palliative treatment, or who presented with rapidly progressing systemic disease. The restricted analysis comprised of 58 patients with a median OS of 8.7 months (95% CI of 5.8 to 11 months) after diagnosis of CNS/LM from NB. There were only three cases of survival beyond three years.

2. Data from 85 patients sourced from The Central German Childhood Cancer Registry, or CGCCR, showed a median OS of 4.7 months. The data was extracted from patients diagnosed between 1990 and 2010. It is estimated that more than 90% of all German childhood cancer patients are registered in this database.

3. Finally, our review of data from 19 patients treated at MSK prior to when ^{131}I-omburtamab was first introduced in 2004, demonstrated a median OS of 5.5 months.

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

Currently, ^{131}I-omburtamab is in pivotal stage development for the treatment of pediatric CNS/LM from NB as a monotherapy after patients have completed standard of care treatment. As of January 2021, 177 patients with pediatric CNS/LM from NB had been treated with ^{131}I-omburtamab in Study 03-133. We are planning to treat a total of at least 32 patients in a multi-center pivotal Phase 2 trial (Study 101) with an interim analysis for the purposes of pharmacokinetic and dosimetry comparability between study sites using ^{131}I-omburtamab from our cGMP commercial manufacturer, versus drug product previously produced by MSK. Study 101 has also been designed to satisfy potential confirmatory study and post-marketing requirement by the FDA, and, as a result, we will continue to recruit a total of at least 32 patients in the study even if we perform an interim analysis before. We expect a BLA re-submission for ^{131}I-omburtamab for CNS/LM from NB via a rolling submission by the end of the second quarter of 2021 or the third quarter of 2021.

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

The trial was originally designed as a Phase 1/2 clinical dose escalation study followed by cohort expansion at the recommended dose. To determine the MTD, patients received up to 70 millicurie, or mCi, ^{131}I-omburtamab in an outpatient setting. Based on treatment result of the 50 mCi dose to treat NB with CNS/LM metastasis and since no DLTs were experienced in the dose escalation part; the 50 mCi dose has been expanded as implemented by a protocol amendment. As of January 2021, 177 patients had been treated with ^{131}I-omburtamab in Study 03-133. Of these, 107 patients were diagnosed with pediatric CNS/LM metastasis from NB. Study 03-133 is now closed for recruitment. We expect that the safety portion of our BLA will be comprised of data from more than 200 patients treated with ^{131}I-omburtamab or ^{124}I-omburtamab across multiple indications.

The table below presents a general clinical overview, including safety data, from Study 03-133 conducted from January 2004 through June 2019. The outlined information in the below table refers to patients treated in Study 03-133.

Omburtamab—Clinical Overview
Study 03-133—Patient Profile and AEs (January 2004 - June 2019)

DIAGNOSIS	No.	Adverse Event (CTC 3.0) Possibly/Probably/ Definite	Percent Myelosuppression
Neuroblastoma	109	Gr 3 or 4 myelosuppression (ANC, hgb, platelets) (88) Gr 4 Hypersensitivity reaction (1) Gr 3 ALT/AST (5) Gr 3 Chemical Meningitis (3) Gr 4 MDS/AML (6) Gr 3 Headache (1)	85%
Medulloblastoma/ PNET	27	Gr 3 or 4 myelosuppression (8) Gr 3 or 4 Chemical meningitis (2) Gr 4 MDS/AML (1) Gr 3 Ataxia (1) Gr 4 Hydrocephalus (1) Gr 4 Encephalopathy (1)	57%
Ependymoma	9	Gr 3 or 4 myelosuppression (3) Gr 3 Headache (1) Gr 2 Nausea/Vomiting (1)	50%
EMTR	4	Gr 3 or 4 myelosuppression (2) Gr 1 Fever (1) Gr 2 Vomiting (1)	50%
Sarcoma	9	Gr 3 or 4 myelosuppression (3) Gr 4 AML (1)	75%
Melanoma	5	Gr 3 Myelosuppression (2) Gr 3 Nausea (1) Gr 3 Hypokalemia (1)	50%
Other (ATRT , choroid plexus ca, ovarian ca, retinoblastoma)	14	Gr 4 MDS/AML (1) Gr 3 Vomiting (1) Gr 3 Headache (1)	
TOTAL	177		

Patient Population

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

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

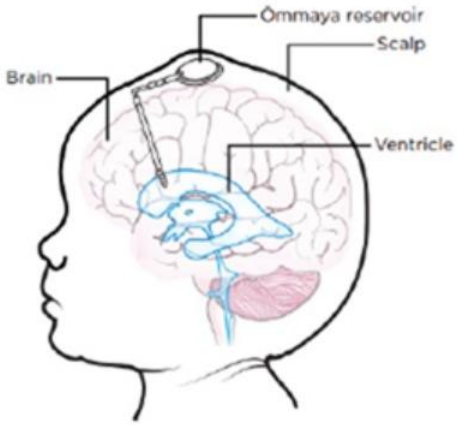
Approximately 80% of all CNS/LM from NB patients presenting at MSK since the initiation of the study were included in the study and the remaining patients were primarily excluded due to the fact that they had already received the maximum dose of previous radiotherapy to CNS, or had progressive systemic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

Treatment Protocol

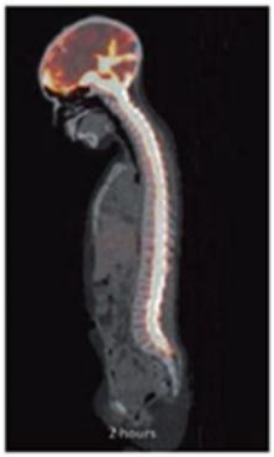
Patients are treated with up to two cycles (each consisting of a treatment dose and a dosimetry dose) of ^{131}I-omburtamab administered through intrathecal infusion via an Ommaya reservoir by which the drug is distributed at the intrathecal space to the entire CSF, (as shown in the figure on the left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

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

Administration of our radiolabeled omburtamab via Ommaya reservoir



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



The diagram on the left depicts how our radiolabeled omburtamab can be administered via the Ommaya reservoir and catheter into the deep ventricles of the brain where the CSF is produced. From the ventricles, our radiolabeled omburtamab will flow with the CSF and spread throughout the entire CNS compartment potentially binding and killing B7-H3 positive cancer cells it may find on its way. The diagram on the right is a PET scan showing the distribution of our radiolabeled omburtamab two hours after administration where it has flowed from the central ventricles throughout the entire CNS compartment.

Primary Objective

- To define the clinical toxicities of intrathecal ^{131}I-omburtamab.

Secondary Objective

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

Safety Results

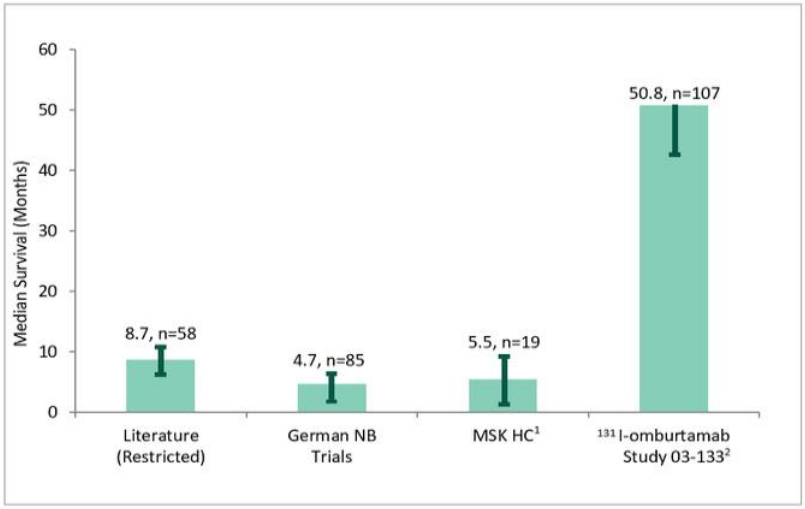
No MTD was reached in the dose escalation portion of the trial. Although not a DLT, myelosuppression was observed in patients who had received craniospinal radiation and ^{131}I-omburtamab at dose levels six and seven (60 and 70 mCi, respectively). As a result, a dose of 50 mCi was chosen for the expansion cohort. Among the 107 patients treated with ^{131}I-omburtamab and diagnosed with pediatric CNS/LM metastasis from NB, a total of 340 injections were administered and myelosuppression was observed in approximately 88 patients. No increased risk of radionecrosis was observed.

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

Efficacy Results

Data reported as of June 2019 indicate that the median OS for the 107 patients diagnosed with pediatric CNS/LM metastasis from NB and treated under Study 03-133 was 50.8 months. Based on calculations per the Kaplan-Meier Plot, the estimated three-year OS of these 107 patients is 56% and the estimated five-year OS and ten-year OS is 44%, and 38%, respectively.

Comparison of Median Overall Survival (Months)



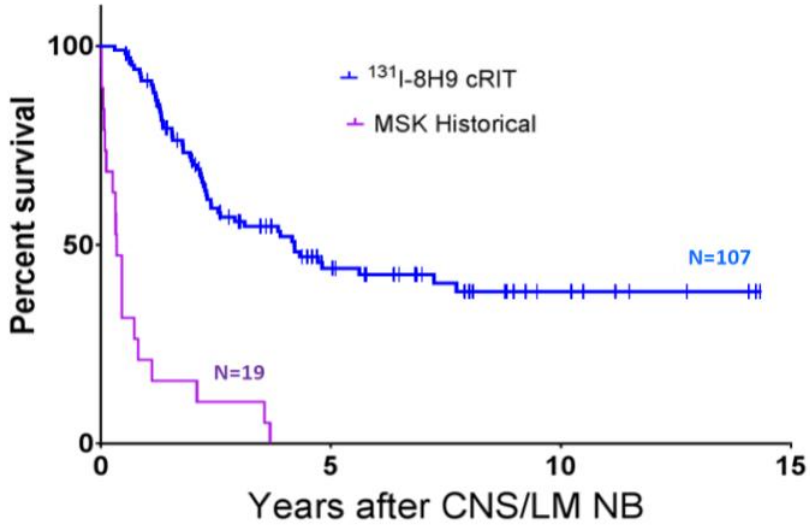
(1) MSK HC = NB patients with CNS / LM treated at MSK prior to 2003.

(2) ^{131}I-omburtamab = Patients diagnosed with pediatric CNS/LM metastasis from NB treated under Study 03-133.

The figure above compares median OS data from Study 03-133 with historical controls. Historical patient data extracted from three sources revealed median OS of 8.7 months in the literature, 4.7 months in the German NB Trials,

and 5.5 months in the MSK historical cohort prior to the introduction of ^{131}I-omburtamab treatment. These results further demonstrate the lack of an established, effective therapy for these patients that we believe can potentially be addressed by ^{131}I-omburtamab.

The chart below shows the historical comparable data and median OS following the introduction of ^{131}I-omburtamab treatment. This represents 107 treated patients from Study 03-133 diagnosed with pediatric CNS/LM metastasis from NB, as at June 2019. The estimated three-year median OS was 56% and the five-year median OS was 44%. Survivors have been followed for up to 14 years.



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

Study 101 is an ongoing pivotal Phase $^{2}/_{3}$ single-arm, open-label, non-randomized, multi-center efficacy, safety, pharmacokinetics and dosimetry trial of intracerebroventricular ^{131}I-omburtamab in pediatric patients with R/R NB who have CNS/LM from NB. Patients will receive up to two cycles of ^{131}I-omburtamab. This study commenced in the second quarter of 2018, and we are planning to treat a total of at least 32 patients in this multi-center pivotal Phase 2 trial with an interim analysis for the purposes of pharmacokinetic and dosimetry comparability between study sites using ^{131}I-omburtamab from our cGMP commercial manufacturer, versus drug product previously produced by MSK. Study 101 has also been designed to satisfy potential confirmatory study and post-marketing requirement by the FDA, and, as a result, we will continue to recruit a total of at least 32 patients in the study even if we perform an interim analysis before. We expect a BLA re-submission for ^{131}I-omburtamab for CNS/LM from NB via a rolling submission by the end of the second quarter of 2021 or the third quarter of 2021.

Data from the planned interim analysis described above will also be combined with the data from Study 03-133 to support a potential accelerated approval for ^{131}I-omburtamab for the treatment of pediatric patients with high-risk NB who have CNS/LM relapse.

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

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

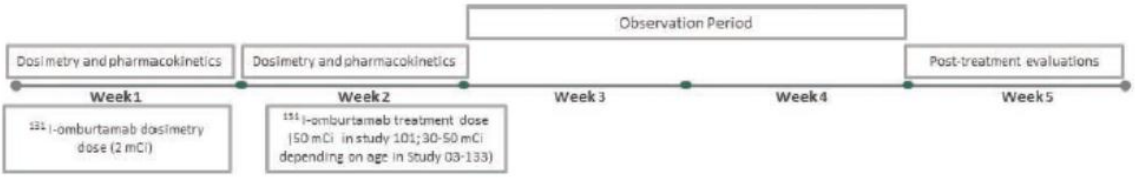
Patient Population

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

Treatment Protocol

A single treatment cycle will last five weeks and will include premedication, intracerebroventricular ^{131}I-omburtamab administration (one dosimetry dose and one treatment dose), an observation period, and post-treatment evaluations (see figure below). The dosimetry dose will be used only for the first 24 patients.

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



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

Primary Objective

- To determine OS rate at three years.

Secondary Objectives

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

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

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

^{124}I-omburtamab is currently being evaluated in an ongoing Phase 1 clinical trial (Study 11-011) for the treatment of DIPG. In contrast to Iodine-131, which emits electrons, Iodine-124 is a positron-emitting iodine isotope. This enables measurement of iodine uptake using PET scans, which we believe is important when using radiotherapy in a critical organ such as pons, where overdosing may have serious consequences. In 2016, ^{124}I-omburtamab received RPDD from the FDA for the treatment of DIPG. As of January 2021, we have treated 46 patients with DIPG with ^{124}I-omburtamab. Interim clinical results from the dose escalation portion of the study, which were published in 2018 (Souweidane et al., Lancet Oncol 2018; 19: 1040-50), demonstrated that convection-enhanced delivery, or CED, of ^{124}I-omburtamab in the brainstem of children with DIPG appears to be a generally feasible approach for drug delivery, based on an evaluation using distribution and pharmacokinetics. We believe that we may qualify for a sBLA, assuming positive pivotal data in the planned Phase 2 trial (Study 102).

Overview of Diffuse Intrinsic Pontine Glioma

DIPG is a highly aggressive, malignant and difficult to treat brain tumor that forms from the glial (supportive) cells of the brain. The tumor grows in the area of the brainstem, called the pons, a critical area of the brain. Pons are involved in regulating critical body functions such as respiration and consciousness. They also house cranial nerves that facilitate essential functions such as eye movements, chewing, swallowing, facial expressions, hearing and balance, and assists in the transmission of messages between the various structures of the brain and the spinal cord.

DIPG typically affects children between the ages of five to nine years old and is the most common brainstem tumor in children, representing 75% to 80% of pediatric brainstem tumors. There are an estimated 300 children diagnosed with DIPG per year in the United States. One published research analysis evaluating DIPG specimens using immunohistochemistry demonstrated that 100% (nine out of nine) of the tested specimens were B7-H3 positive.

While DIPG accounts for approximately 10% to 15% of brain tumors in the pediatric population, it constitutes approximately 80% of brain tumor-related deaths. Assuming similar prevalence as in the United States, we estimate approximately 450 new pediatric patients diagnosed with DIPG per year in Europe. We believe the current addressable market for DIPG consists of approximately 750 new pediatric DIPG patients each year in the United States and Europe, combined.

^{124}I-omburtamab for Diffuse Intrinsic Pontine Glioma—Current Treatment Landscape and Associated Limitations

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

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

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

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

Study 11-011: A Phase 1 Study of Convection-Enhanced Delivery of ^{124}I-omburtamab for Patients with Non-Progressive Diffuse Pontine Gliomas Previously Treated with External Beam Radiation Therapy

MSK is conducting a Phase 1, dose escalation study of convection enhanced delivery, or CED, of ^{124}I-omburtamab in children with non-progressive DIPG previously treated with external beam radiation therapy. The study commenced in December 2011 and as of January 2021, 46 patients had been enrolled. We expect to enroll a total of 56 patients.

Patient Population

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

Treatment Protocol

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

Primary Objective

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

Secondary Objectives

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

Safety Results

As noted above, data was published in Lancet Oncology (Souweidane et al., 2018) which demonstrated that CED appears to be a feasible approach for drug delivery in the brainstem of children with DIPG as evaluated by assessment of the distribution of the infusate and pharmacokinetics. As of November 2020, 46 patients had been enrolled. In Study 11-011, 8 subjects reported AEs of CTCAE Grade 3 considered related to ^{124}I-omburtamab. These were generally indicative of nervous system effects of radiation necrosis such as hemiparesis (3 subjects), dysarthria (3 subjects), ataxia (3 subjects), dysphagia (2 subjects), muscular weakness (2 subjects) and gait disturbance (1 subject). The remaining Grade 3, related events were skin infection, anxiety, and stridor (1 subject each). There were no related AEs CTCAE grade 4 or 5.

Multi-Center Study

We and the principal investigator for Study 11-011 are currently drafting a pivotal Phase 2 study based on the experiences from Study 11-011. This study will be a non-randomized, multi-center, Phase 2 trial using CED of ^{131}I-omburtamab to the brainstem of children with DIPG. Each patient will have previously received external beam radiotherapy to the brainstem and will not have shown clear evidence of tumor progression following this radiotherapy. Diagnostic and eligibility decisions for patients entering the study will be made by a multidisciplinary pediatric neuro-oncology team at the treating site. Eligibility and surgical planning will be centrally reviewed. Patients may undergo up to three repeats of treatment using CED of ^{131}I-omburtamab. MRI and SPECT will be used for confirmation of appropriate drug distribution patterns. The primary endpoint will be OS and the secondary endpoints includes PFS and monitoring of distribution patterns using advanced MR-based algorithms. Perioperative morbidity, device performance (catheter for antibody delivery in pons), and patient tolerance after CED treatment will be monitored. Liquid biopsies (plasma and CSF samples) will be explored for ctDNA content as a correlate of tumor response.

^{131}I-omburtamab for Treatment of Desmoplastic Small Round Cell Tumor

^{131}I-omburtamab has been evaluated in a Phase 1 clinical study (Study 09-090) for the treatment of DSRCT. In the data from 48 patients presented in November 2019, no DLTs were observed and a MTD was not reached. In addition, there was no significant myelosuppression and stem cell rescue was not required. We believe that we may qualify for a sBLA, assuming positive pivotal data. A Phase 2 study (Study 19-182) for the treatment of DSRCT is also ongoing.

Overview of Desmoplastic Small Round Cell Tumor

DSRCT is a rare and aggressive type of a soft tissue cancer (sarcoma) that primarily affects children and young adults and is more common in males. It is formed by small, round cancer cells surrounded by scar-like tissue and is often found in the peritoneum (the tissue that lines the inside of the abdomen and pelvis). Most patients present with abdominal or pelvic tumors, with subsequent metastases to distant lymph nodes, BM and lungs. Due to the rarity of this neoplasm, no large population based studies exist. Analysis presented in literature suggests there are approximately 100 patients diagnosed with DSRCT per year in the United States. Assuming similar prevalence as in the United States, we estimate approximately 150 patients diagnosed with DSRCT per year in Europe. A published report examining DSRCT samples using immunohistochemistry showed that 44 of 46 (or 96%) of tumor samples were B7-H3 positive. We believe the current addressable market for DSRCT consists of approximately 160 new DSRCT patients each year, representing approximately 65% of all new patients diagnosed with DSRCT in the United States and Europe, combined.

^{131}I-omburtamab for Desmoplastic Small Round Cell Tumor—Current Treatment Landscape and Associated Limitations

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

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

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

^{131}I-omburtamab is being evaluated from a completed clinical Phase 1 study (Study 09-090) for the treatment of DSRCT. A Phase 2 study (Study 19-182) for the treatment of DSRCT is ongoing. We intend to discuss the protocol for this study (Study 19-182) with the FDA and we believe that we may qualify for a sBLA, assuming positive pivotal data.

Study 09-090: Phase 1 Study of Intraperitoneal Radioimmunotherapy with ^{131}I-omburtamab for Patients with Desmoplastic Small Round Cell Tumors and Other Solid Tumors Involving the Peritoneum

MSK has completed a clinical study of IP ^{131}I-omburtamab for treatment of patients with DSRCT and other B7-H3 positive solid tumors metastatic to the peritoneum. The primary purpose of the study is to define the toxicity and the MTD, assess the pharmacokinetics, and assess response of DSRCT and other solid tumors. The study has completed accrual and 47 patients have been exposed. Data from this study was presented at the 2019 Connective Tissue Oncology Society annual meeting in November 2019 based on an evaluation of 33 Gross Total Resection, or GTR, patients treated at MSK from 2009 to 2017. A total of 24 patients received Whole Abdominalpelvic Intensity-Modulated RadioTherapy, or WA-IMRT, in combination with omburtamab Interperitoneal Radio Immunotherapy, or IP-RIT, and nine patients received WA-IMRT without omburtamab IP-RIT. The study showed a median OS of 41 months for the DSRCT patients who did not receive omburtamab IP-RIT and 59 months for those receiving omburtamab IP-RIT. The data indicates that adding IP-RIT with iodinated omburtamab to the standard WA-IMRT treatment appears to be well tolerated.

Patient Population

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

Treatment Protocol

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

Primary Objective

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

Secondary Objectives

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

Safety Results

In the data from 48 patients with DSRCT presented in November 2019, no DLTs were observed and a MTD was not reached. In addition, there was no significant myelosuppression and stem cell rescue was not required. Toxicity was overall low-grade and transient and mainly myelosuppression. No events of febrile neutropenia. We believe that the initial data from the first group of patients supports continued investigation of the benefit of ^{131}I-omburtamab in this patient population.

Study 19-182: A Phase 2 Study - Combination of ^{131}I-omburtamab Radioimmunotherapy and External Beam Radiotherapy for Desmoplastic Small Round Cell Tumors and Other Solid Tumors involving the Peritonuem

This is an ongoing Phase 2 clinical study of IP RIT with ^{131}I-omburtamab plus WA-IMRT for patients with DSRCT who have undergone gross total resection, or GTR, of their abdominopelvic disease (as assessed by the operating surgeon and documented in the OR note) and who have no definitive radiological evidence of disease in liver or outside the abdomen/pelvis (Group A). Patients with DSRCT without GTR (Group B); and patients with tumors other that DSRCT who are B7H3-positive on immunohistochemistry (Group C) will receive only IP ^{131}I- omburtamab.

As of January 2021, 7 patients have been enrolled in this study.

All patients will be administered a single dose of IP RIT administered through an IP catheter with ^{131}I-omburtamab at a dose of 80mCi/m2. ^{131}I-omburtamab pharmacokinetics will be studied by a blood draw from indwelling venous lines. ^{131}I-omburtamab biodistribution will be studied by a single gamma camera scan 5-7 days after IP injection where feasible. Thyroid protection is commenced at least 7 days prior to administration of the ^{131}I-omburtamab dose and continued for 28 days after. Group A patients will receive WA-IMRT approximately 2-4 weeks after completing IP-RIT. A dose of 30 Gy will be delivered in 20 fractions of 1.5 Gy given once daily, 5 days per week over the course of approximately 4 weeks.

Non-Clinical Safety

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

^{177}Lu-omburtamab-DTPA Overview

We intend to leverage our expertise with omburtamab to develop product candidates for the treatment of indications associated with pediatric and large adult patient populations. We believe that our clinical experience with ^{131}I-omburtamab in 41 patients with tumors such as sarcoma, melanoma and medulloblastoma supports this objective. Our first such product candidate targeted towards larger patient populations is DTPA-conjugated omburtamab radiolabeled with Lutetium-177, which is currently in Phase 1 clinical development for the treatment of medulloblastoma and B7-H3 positive LM from solid tumors.

Animal toxicity studies of ^{177}Lu-omburtamab have been completed on current Good Laboratory Practices, or GLP, material and cGMP production has been established. Diethylenetriamine pentaacetate, or DTPA, is an organic molecule that acts as a chelator of metals such as Lutetium. DTPA can bind to radioactive materials to decrease the amount of time it takes to flush the radioactive material from the body. The resulting product candidate, omburtamab-DTPA-Lutetium-177 conjugate, or ^{177}Lu-omburtamab-DTPA, can be distributed directly to hospitals, already conjugated and ready to use. It may then be administered to patients as a single-step push dose via an indwelling catheter for intracerebroventricular drug delivery, similar to the administration of ^{131}I-omburtamab in CNS/LM from NB. Therefore, we believe that a more easily available ready to use radiolabeled antibody such as ^{177}Lu-omburtamab-DTPA could be used more frequently, thereby significantly expanding our patient population beyond children.

On December 31, 2019, we submitted an Investigational New Drug application, or IND, for ^{177}Lu-omburtamab-DTPA, for the treatment of medulloblastoma and we opened the IND for patients in October 2020. We expect to enroll the first patient in this Phase 1 trial in the first quarter of 2021. We have also opened a Phase 1 with ^{177}Lu-omburtamab-DTPA targeting B7-H3 positive CNS/LM tumors in adults, and expect to enroll the first patient in this trial in the first quarter of 2021.

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

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

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

Despite aggressive treatment, CNS/LM has a poor prognosis with less than 15% of all patients surviving one year following diagnosis. The median OS of untreated patients with CNS/LM is four to six weeks. The median OS of patients with combined treatment (often comprising surgery, radiation and/or chemotherapy) is usually less than eight months.

The incidence of CNS/LM is increasing. An important factor contributing to the increasing incidence of CNS/LM is the availability of more effective systemic therapies. These therapies may increase survival time and could therefore lead to a higher incidence of metastatic disease.

^{177}Lu-omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases—Current Treatment Landscape and Associated Limitations

Treatment of most patients with CNS/LM requires a combination of surgery, radiation, and/or chemotherapy. However, CNS/LM has proven difficult to treat due to the localization of the tumor within the leptomeningeal surfaces of the CNS compartment making complete removal by surgery difficult. Moreover, the blood-brain barrier, a membrane that selectively regulates molecules entering the brain from the blood, often inhibits drug delivery to the brain due to the inability of large molecules to cross the blood-brain barrier. Because the most common tumors that metastasize to the brain express B7-H3, in contrast with normal brain tissue that lacks B7-H3 expression, we believe that the incidence of B7-H3 expression makes omburtamab a viable antibody for targeting metastatic tumors in the CNS.

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

We are developing a Lutetium-177 conjugated omburtamab with DTPA as chelator. 177Lu-omburtamab-DTPA will be administered to patients as a single-step push dose via an indwelling catheter for intracerebroventricular drug delivery, similar to the administration of ^{131}I-omburtamab in CNS/LM from NB. This form of administration will allow us to bypass the blood brain barrier and gain direct access to the CNS/LM. Lutetium-177 is a medium-energy beta-emitter with a maximal tissue penetration of 2 mm. Its half-life is approximately 6.7 days. Lutetium-177 also emits low-energy Gamma rays, which allows scintigraphy and subsequent dosimetry with the same therapeutic compound. Lutetium-177 is bound to omburtamab by DTPA. The resulting product ^{177}Lu-omburtamab-DTPA conjugate can be distributed ready to use. Lutathera, a Lutetium-177-DOTA conjugated somatostatin analogue peptide, has already demonstrated significant clinical efficacy in patients with progressive neuro endocrine tumors, or NETs, and is approved by the EMA and the FDA, in this orphan indication. In a multi-center, randomized, comparator-controlled, parallel-group Phase 3 study that has been the basis for regulatory submission for Lutathera, it demonstrated a significant improvement in PFS in patients with inoperable progressive midgut NETs compared to the general standard of care, with limited acute toxic effects. The beta radiation of Lutetium-177 is similar to the beta radiation emitted from radioactive iodine, which already has demonstrated efficacy in CNS/LM from NB when conjugated to omburtamab.

We believe Lutetium-177 may have a number of potential advantages over both Iodine-131 and Iodine-124. In particular, the radiolabeling of omburtamab with Lutetium-177 involves a relatively simple one-step procedure and can be distributed conjugated ready to use.

Study 302: ^{177}Lu-omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases from solid tumors in adults - Clinical Development Program

The study is designed as an open label single arm, first in human, dose escalation and expansion study to evaluate safety and efficacy of ^{177}Lu-omburtamab-DTPA in adult patients with Central Nervous System/Leptomeningeal Metastases from solid tumors with B7-H3 expression. We plan to enroll up to 72 patients in this Phase 1/2 study which we anticipate to initiate in the first quarter of 2021 following the FDA clearance of the IND in October 2020.

Primary Objective

- To explore the tolerability of ^{177}Lu-omburtamab-DTPA

Secondary Objectives include

- To assess absorbed radiation doses and organ dosimetry for ^{177}Lu-omburtamab-DTPA
- To evaluate the investigator assessed response, PFS and OS

Study 301: ^{177}Lu-omburtamab-DTPA in medulloblastoma - Clinical Development Program

The study is designed as an open label single arm, first in human, dose escalation and expansion study to evaluate ^{177}Lu-omburtamab-DTPA in patients with R/R medulloblastoma. We plan to enroll approximately 49 patients in this Phase 1/2 study which we anticipate to initiate in the first quarter of 2021 following the FDA clearance of the IND in October 2020.

Primary Objective

- To explore the tolerability of ^{177}Lu-omburtamab-DTPA.

Secondary Objectives include

- To assess absorbed radiation doses and organ dosimetry for ^{177}Lu-omburtamab-DTPA
- To evaluate the investigator assessed response PFS and OS

Humanized Omburtamab Overview

We are also developing huB7-H3, a humanized version of omburtamab, for the treatment of B7-H3 positive adult solid tumors where systemic immunotherapy is needed. We expect that huB7-H3 will be used as a radio-conjugated antibody designed to overcome limitations of murine antibodies that may induce HAMA, which may lead to decreased efficacy and increased toxicity when used for systemic immunotherapy.

Bispecific Antibody Programs Overview

We are advancing a promising pipeline of novel bivalent tumor targeting BsAbs for the treatment of cancer. We believe that our BsAbs have the potential to overcome limitations associated with existing BsAb constructs. Our first BsAb clinical product candidate, nivatrotamab, is a humanized anti-GD2 and anti-CD3 BsAb. We have successfully opened an IND for our Phase 2 trial with nivatrotamab in Small Cell Lung Cancer. In addition a Phase 1/2 trial with nivatrotamab, for the treatment of refractory GD2 positive adult and pediatric solid tumors is ongoing.

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

In addition, the MSK License provides us with non-exclusive access to MSK's technology that facilitates the creation of a novel human protein tag that can dimerize, or link together, BiTEs, which we refer to as the MULTI-TAG technology platform. BiTEs are an important class of BsAbs that has shown significant promise in the treatment of cancer due to their high potency. Based on our pre-clinical studies, we believe that this novel class of BiTEs has the potential to result in better tumor-binding, longer serum half-life and significantly greater T-cell mediated killing of tumor cells without the need for continuous infusion.

Overview of Current Bispecific Antibody Treatment Approaches

BsAbs are engineered proteins capable of simultaneously binding to two different epitopes, on the same or different antigens. Through simultaneous recognition of two different targets, BsAbs can serve as mediators for the redirection of immune effector cells, such as Natural Killer cells, or NK cells, and T-cells, to tumor cells, in order to enhance tumor cell destruction. In addition, by targeting two different receptors in combination on the same cell, BsAbs can induce modifications of cell signaling, including the inactivation of pathways. BsAbs represent an exciting approach to cancer immunotherapy because, among other factors, they have the potential to overcome the limitations of conventional monoclonal antibody approaches to treating cancers. Moreover, BsAbs can be mass produced without the manufacturing complications and risk of persistent systemic toxicity associated with other new immunotherapy approaches such as CAR-T therapy.

BsAbs are generally divided into two classes, IgG-like molecules and non-IgG-like molecules. IgG-like BsAbs retain the traditional monoclonal antibody structure but bind to multiple antigens. Although IgG-like BsAbs generally demonstrate adequate stability and effector functions, their large size limits tissue penetration.

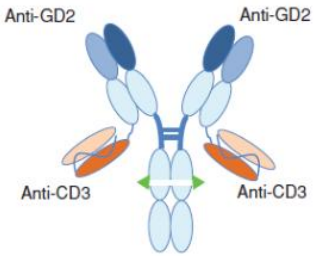
Non-IgG-like BsAbs lack a fragment crystallizable, or Fc, region, consisting instead of chemically linked variable regions and various types of multivalent single-chain variable fragments, or scFvs. One type of non-IgG-like BsAbs are BiTEs. BiTEs are relatively small and have more efficient penetration, however, they exhibit short serum half-lives. They bind monovalently to tumor targets, which often results in suboptimal tumor binding relative to our conventional IgG-like BsAbs that bind bivalently. Finally, therapeutic dosing of BiTEs is limited by the risk of excessive cytokine release in patients.

The only approved BsAb for treatment of cancer in the United States is blinatumomab, a BiTE, approved for the treatment of acute lymphocytic leukemia.

Nivatrotamab Overview

The figure below depicts our first BsAb product candidate, HuGD2-BsAb, or nivatrotamab, a fully humanized IgG-scFv format antibody, in which the anti-CD3 scFv is linked to the carboxyl end of the naxitamab IgG1 and the Fc region is mutated to help prevent cytokine release as well as complement-mediated pain side effects.

Nivatrotamab (anti-GD2 and anti-CD3)

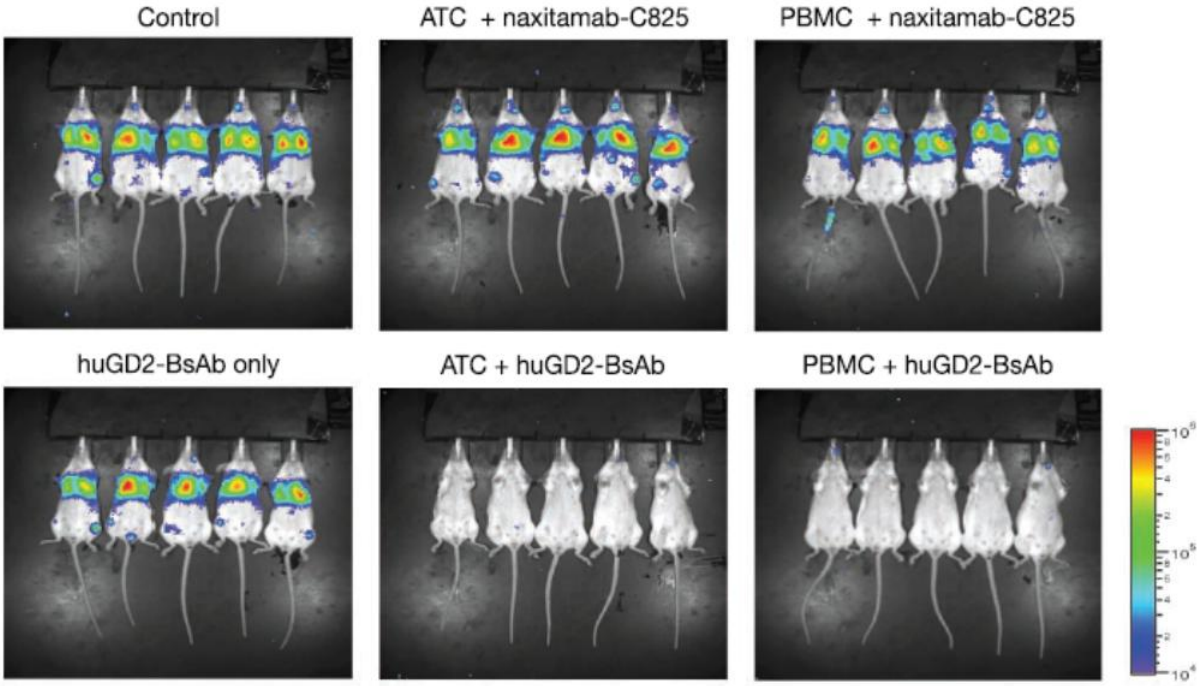


We believe that nivatrotamab may have several potential advantages over other BsAbs, including:

- Improved potency due to bivalency towards GD2, while maintaining functional monovalency towards CD3.
- Longer serum half-life (as compared to e.g. BiTE) to improve efficacy and patient convenience—molecular size of 210kD (vs. 55kD size of blinatumomab) and binding to neonatal Fc receptor result in longer serum half-life, thereby reducing the need for continuous infusion.
- Better safety profile:
    - The larger size of our molecule prevents leakage into the CNS thereby avoiding CNS neurotoxicity; and
    - Low affinity for CD3 molecules and functional monovalency towards CD3 reduces risk of significant cytokine release.

Knockout mice, which lack murine T-cells, B-cells and NK cells, were used for human cancer xenograft studies. The picture below demonstrates a study where mice were transplanted with human M14-Luc melanoma and human peripheral blood mononuclear cells, or PBMC, or activated T-cells, or ATC, as effector cells. Tumor growth was assessed by luciferin bioluminescence.

Mice, in a control group, treated with saline without effector cells (nivatrotamab only), or effector cells plus ATC+naxitamab-C825, used as the control BsAb and which does not bind to T-cells, had equally rapid tumor progression. In contrast, mice treated with nivatrotamab (huGD2-BsAb) in the presence of human effector cells (ATC+huGD2-BsAb or PBMC+huGD2-BsAb) demonstrated nearly total tumor elimination. The picture below is a representative image at day 31.



On December 10, 2018, the FDA cleared the IND application for nivatrotamab, and in January 2019, a Phase 1/2 trial was initiated at MSK for the treatment of refractory GD2 positive adult and pediatric solid tumors.

Study 18-034: Phase 1/2 study of humanized 3F8 bispecific antibody (Hu3F8-BsAb) in patients with relapsed/refractory neuroblastoma, osteosarcoma, and other GD2(+) solid tumors

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

On January 15, 2021, the FDA cleared a second IND application for nivatrotamab in relapsed/recurrent metastatic small-cell lung cancer (Study 402).

Study 402: Safety and clinical activity of nivatrotamab, an anti GD2×CD3 bispecific antibody, in relapsed/recurrent metastatic small-cell lung cancer

Study 402 is a multicenter Phase 2 single arm, dose escalation clinical trial of nivatrotamab in relapsed/recurrent metastatic SCLC. Dose escalation is performed in up to 35 patients in Part 1 of the study and, in addition, approximately 30 patients will be enrolled in Part 2 of the study. Part 1 endpoints include MTD, RP2D, safety, PK, HAHA, and anti-tumor activity. For Part 2, the endpoint will include safety at RP2D, anti-tumor activity (ORR, DCR, duration of response, PFS and OS), and HAHA. Currently, no published safety data is available for this study.

CD33-BsAb Overview

Our second BsAb product candidate, CD33-BsAb, is a humanized anti-CD33 and anti-CD3 BsAb for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage. Currently we are planning to set up GLP and cGMP production allowing for potential IND filing in 2021.

GD2-GD3 Vaccine Overview

Neuroectoderm-derived tumors, including NB and sarcomas, have high expression of tumor antigens GD2 and GD3. Our investigational bivalent GD2-GD3 Vaccine is being studied in a clinical Phase 2 study (Study 05-075) conducted at MSK for the immunization of high-risk NB patients previously treated with DANYELZA. The vaccine, in combination with adjuvants, is being studied to induce patients to produce their own anti-GD2 and anti-GD3 serum titers, with the goal of preventing subsequent relapse.

GD2-GD3 Vaccine —Clinical Development Program

A Phase 2 study (Study 05-075) of the GD2-GD3 Vaccine with the immunological adjuvant OPT-821, in combination with oral ß-glucan for high-risk NB is being conducted at MSK. MSK has enrolled more than 260 patients in this study.

We are planning a multicenter, Phase 2 clinical study in relapsed, high-risk NB patients after obtaining complete response on salvage therapy. We plan to open this study in 2021.

MULTI-TAG Technology Overview

We believe that our non-exclusive access to the MULTI-TAG technology will help us make further advances to our BsAb program by optimizing BiTEs. While there has been significant enthusiasm for BiTEs given their high potency and ability to penetrate more efficiently than conventional IgG-like BsAbs, their efficacy remains hampered by their size and binding characteristics. BiTEs are relatively small in size, approximately 55kD, resulting in a short serum half-life given rapid renal clearance. As a result, they require continuous infusion for several weeks in order to achieve a therapeutic response. They also bind monovalently, which often results in suboptimal tumor binding. Further, therapeutic dosing of BiTEs is limited by the risk of excessive cytokine release in patients.

Using the MULTI-TAG technology, we have designed a novel protein tag of human origin that dimerizes, or links, BiTEs, in a unique conformation, which we believe may result in improved tumor binding, a longer half-life, and greater T-cell mediated tumor cell killing. We are using the MULTI-TAG technology platform to dimerize our BsAbs into proteins of approximately 120kD in size, thereby increasing serum half-life without the need for continuous infusion. The unique dimerized conformation, while binding bivalently to tumors, also binds monovalently to T-cells, which we believe, leads to limiting excessive cytokine release.

SADA Technology - Liquid Radiation™

The SADA technology's 2-step payload delivery can be achieved in an in-vitro setting, where tumors have been shown to shrink or completely disappear, while other tissues were spared. No clearing agent is needed, and no significant toxicity to bone marrow, kidneys or liver tissues has been observed. We believe that the SADA technology may allow for rapid clearance of the compound, while maintaining high target uptake, and thereby causing less immunogenicity. In addition, the SADA technology appears to be modular, whereby any DOTA-modified radioactive payload combined with any therapeutic antibody seems possible.

We are using the SADA Technology to advance a series of antibody constructs based on the SADA technology, where bispecific antibody fragments bind to the tumor before a radioactive payload is injected in a two-step approach. We also refer to the SADA technology as Liquid Radiation™. We have designated GD2-SADA for potential use in GD2 positive solid tumors, B7-H3-SADA for potential use in prostate cancer, GPA33-SADA for potential use in colon cancer, and HER2-SADA for potential use in breast cancer as our first SADA constructs and expect to file an IND for

GD2-SADA in 2021. We believe the SADA technology could potentially improve the efficacy of radiolabeled therapeutics in tumors that have not historically demonstrated meaningful responses to radiolabeled agents.

Manufacturing

Currently, we contract with third party cGMP vendors for the manufacturing of our product candidates for pre-clinical studies, clinical trials and commercial supply. We do not currently own or operate any manufacturing facilities to produce clinical or commercial quantities of our product candidates. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. To meet our projected needs for commercial manufacturing third parties with whom we currently work may need to increase their scale of production or we may need to secure alternate suppliers. Although we rely on our cGMP manufacturers, we have personnel with substantial manufacturing experience to oversee our relationships with such manufacturers.

Manufacturing clinical and commercial products is subject to extensive regulations that impose various procedural and documentation requirements, which govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance. Our vendors are required to comply with cGMP regulations, which are regulatory requirements enforced by the FDA and other regulatory bodies like the EMA to assure proper design, monitoring and control of manufacturing processes and facilities for human pharmaceuticals.

Our current product candidates are mainly manufactured based on well-established technology known from mAb products. These manufacturing processes involve the genetic engineering of a parental host cell line to isolate a cell that produces the target product. Once a cell line is isolated, a cell bank is produced under prescribed and documented conditions. The cell bank, preserved frozen, is tested, as required by regulations, to demonstrate that the engineered cell line is free from potentially harmful impurities and contaminants, such as viruses.

The drug substance is an active ingredient that is intended to furnish pharmacological activity or other direct effect in the diagnosis, cure, mitigation, treatment, or prevention of disease or to affect the structure or any function of the human body. The manufacturing process for the drug substance begins with the thaw of vials from the cell bank and growth of these cells in established media until sufficient cells are cultured to inoculate a production bioreactor. The cells in the production bioreactor are grown in chemical defined media and under controlled and monitored conditions that stimulate the production of the antibody into the culture media. The production bioreactor is cultured for an established period of time and is then harvested by filtration to remove the cells from the culture media.

The solution containing the product is purified through several steps to remove known process- and product-derived impurities. The technologies employed include ultrafiltration and column and membrane chromatography. Additional steps are performed to inactivate or remove viruses. The final step of the drug substance process adjusts the antibody concentration and produces the final formulation to be used for drug product production. The drug substance is tested to meet pre-established product specific release criteria for purity, potency and safety, and is then periodically tested to demonstrate stability upon storage as required by regulations. The drug substance is stored at prescribed temperatures, typically refrigerated or frozen.

The drug product is produced by sterilization filtration of the drug substance solution, followed by aseptic filling into glass vials and then stoppered. The drug product is subjected to product specific release testing for purity, potency and safety according to pre-established specifications. Drug product lots are periodically tested to demonstrate stability over the established storage expiry period. The drug product is stored and shipped under temperature-controlled conditions, typically refrigerated, to sites designated for clinical trial testing, or eventually to commercial pharmaceutical logistics providers.

DANYELZA is a recombinant humanized IgG1κ monoclonal antibody against GD2 expressed in Chinese Hamster Ovary, or CHO, cells. A one mL ampoule from the cell bank is used to establish material for seeding of 1,000 L fed batch bioreactor in chemical defined media with no animal derived component. After growth of the cells are completed the un-processed bulk from the bioreactor containing the DANYELZA drug substance undergoes clarification by filtration, and subsequent multi-step product purification. The DANYELZA drug substance is manufactured by Patheon Biologics B.V. in Groningen, The Netherlands and the DANYELZA drug product is manufactured at Patheon

Manufacturing Services LLC in Greenville, North Carolina, (both part of the Thermo Fisher Scientific Inc., group of companies) collectively Patheon/Thermo Fisher. All manufacturing activities are performed in compliance with cGMP regulations and no excipients of human or animal origin have been used. The DANYELZA drug product is packaged in 10 mL ISO 10R glass vials and refrigerated.

Omburtamab is a murine IgG1 monoclonal antibody against B7-H3. The antibody is manufactured in a 200 L bioreactor in chemical defined media with no animal derived components. After clarification of the fermentation and a multi-step purification process as well as packaging, the drug substance is ready for filling. The non-radiolabeled omburtamab is filled in 2 mL ISO 2R glass vials and frozen and is ready for radiolabeling. The drug substance is manufactured by EMD Millipore Corporation (now part of the Merck KgaA group of companies), or EMD/Merck, in Martillac, France, and the non-radiolabeled omburtamab drug product is manufactured by Patheon/Thermo Fisher in Ferentino, Italy. Radiolabeling with Iodine-131 is performed at SpectronRx in South Bend, Indiana, USA, and final product is delivered for clinical trial or also later commercial supply, if approved.

While we believe that Patheon/Thermo Fisher, EMD/Merck and SpectronRx are capable of producing sufficient quantities of drug product to support our clinical and commercial supply for DANYELZA and omburtamab, we also believe that there are a number of alternative third-party manufacturers that have similar capabilities that would be capable of providing sufficient quantities of drug product. However, should Patheon/Thermo Fisher, EMD/Merck and/or SpectronRx not be able to provide sufficient quantities of drug product for our planned clinical trials or commercial sales, we would be required to seek and then qualify another contract manufacturer to provide this drug product, likely resulting in a delay in such trials and loss of, or delayed, commercial sales.

Our GD2-GD3 Vaccine is manufactured from natural sources and processed by chemical conjugation, lactonization and freeze drying into the final drug product. Currently the GD2-GD3 vaccine is manufactured at Magle Chemoswed AB in Malmoe, Sweden.

Commercialization Plan

The sales call points for DANYELZA and our late-stage product candidates in the United States and the European Union are highly concentrated. This enables us to effectively service our customers and call points with a small commercial organization. Both our targeted commercial go to market approach as well as our partnership with MSK, have already afforded us the opportunity to identify patients for DANYELZA and our product candidates, if approved. We believe these factors position us well for commercialization.

Our management team understands the complexity of rare oncological diseases and we believe we have the necessary expertise to be a true partner to patients, caregivers, and advocacy and healthcare teams leading to shared success. As we advance our product pipeline to address larger patient populations, we intend to expand our specialty sales force and continue the development of our organizational infrastructure to support the network of relevant hospitals, cancer centers, oncologists and other physicians as well as continue to provide support to patients, care givers and other healthcare providers. We plan to commercialize our future product candidates in the United States and Europe ourselves, and will continue to evaluate strategic collaborations in select territories in order to maximize the potential of our product candidates.

As additional product candidates advance through our pipeline, our commercial plans may change. The size of the development programs, size of the target market, size of a commercial infrastructure, and manufacturing needs may all influence our strategies in the United States, the European Union and other parts of the world.

Commercialization Partnerships

After the approval of DANYELZA by the FDA, we have entered a number of strategic collaborations in the form of partnerships with select companies to maximize the potential value for the Company. In November 2020, we entered into an exclusive license and distribution agreement for DANYELZA and omburtamab with Takeda Israel, a wholly owned subsidiary of Takeda Pharmaceutical Company Limited covering the State of Israel, West Bank and Gaza Strip. In December 2020, we entered into a distribution agreement for DANYELZA and omburtamab with Swixx

BioPharma AG for the Eastern European territories Bosnia & Herzegovina, Bulgaria, Croatia, Czech Republic, Estonia, Hungary, Latvia, Lithuania, Poland, Romania, Russia, Serbia, Slovakia and Slovenia. Finally, later in December 2020, we entered into a license agreement for DANYELZA and omburtamab with SciClone Pharmaceuticals International Ltd., for Greater China, including Mainland China, Taiwan, Hong Kong and Macau. Together with SciClone, we plan to initiate regulatory activities in the third quarter of 2021.

Competition

The biotechnology and pharmaceutical industries generally, and the cancer drug sector specifically, are characterized by rapidly advancing technologies, evolving understanding of disease etiology, intense competition and a strong emphasis on intellectual property. While we believe that our product candidates and our knowledge and experience provide us with competitive advantages, we face substantial potential competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions and governmental agencies and public and private research institutions.

In addition to the current standard of care for patients, commercial and academic clinical trials are being pursued by a number of parties in the field of immunotherapy. Early results from these trials have fueled continued interest in immunotherapy, which is being pursued by several biotechnology companies as well as by large pharmaceutical companies. Many of our current or potential competitors, either alone or with their collaboration partners, have significantly greater financial resources and expertise in research and development, manufacturing, pre-clinical studies, conducting clinical trials, and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Specifically, MacroGenics, Inc. and Daiichi Sankyo Co. are developing antibodies against B7-H3. United Therapeutics Corporation, or United Therapeutics, has commercialized Unituxin (dinutuximab), an antibody against GD2, in the United States and has announced plans to seek a label expansion for Unituxin in combination with irinotecan and temozolomide for the treatment of pediatric patients with R/R NB. During the third quarter of 2020, United Therapeutics discontinued its efforts to investigate Unituxin's potential activity against adult cancerous tumors, and its efforts to develop a humanized version of Unituxin. In addition, DANYELZA 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. 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. EUSA has previously announced plans to file for registration of dinutuximab beta in the United States in 2020 in R/R NB. In January 2020, EUSA and BeiGene Ltd., or BeiGene, announced an exclusive collaboration to commercialize Qarziba® in mainland China and in November 2020 EUSA and BeiGene announced that the BLA for QARZIBA® (Dinutuximab beta) was accepted by the China National Medical Products Administration and granted priority review.

Intellectual Property

Patent Portfolio

We strive to protect and enhance the proprietary technology, inventions, and improvements that we believe are commercially important to our business, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from our collaborators or other third parties. Our policy is to seek to protect our proprietary position by, among other methods, filing patent applications in the United States and in jurisdictions outside of the United States related to our proprietary technology, inventions, improvements, and product candidates that are important to the development and implementation of our business. We also rely on trade secrets and know-how relating to our proprietary technology and product candidates, continuing innovation, and in-licensing opportunities to develop, strengthen, and maintain our proprietary position in the field of immunotherapy. We additionally rely on data

exclusivity, market exclusivity, and patent term extensions when available, and plan to seek and rely on regulatory protection afforded through orphan drug designations. Our commercial success will depend in part on our ability to obtain and maintain patent and other proprietary protection for our technology, inventions, and improvements, whether developed internally or licensed from our collaborators or other third parties; to preserve the confidentiality of our trade secrets; to maintain our licenses to use intellectual property owned by third parties; to defend and enforce our proprietary rights, including our patents; and to operate without infringing on the valid and enforceable patents and other proprietary rights of third parties.

We have in-licensed numerous patents and patent applications and substantial know-how relating to the development and commercialization of our immunotherapy product candidates, including related manufacturing processes and technology. In addition, an international patent application has been filed claiming the inventions of investigators at MSK as well as personnel of Y-mAbs Therapeutics.

As of December 31, 2020, our patent portfolio included:

- For our DANYELZA patent portfolio, we have an exclusive license from MSK to MSK's rights in two patent families. The first family consists of patents and patent applications with composition of matter claims covering humanized or chimeric antibodies or fragments thereof comprising specific sequences and capable of binding to GD2, and includes three U.S. patents, one Australian patent, two New Zealand patents, one Chinese patent, one Japanese patent, one South Korean patent, one Hong Kong patent, one Indian patent, one Canadian Patent and one pending patent application in Europe. We expect that any patents that issue in this first family will expire in June 2031. A core U.S. patent in this family is expected to expire on June 20, 2031. The second family consists of applications with composition of matter claims covering high affinity anti-GD2 antibodies, and includes one US patent, one German patent, one French patent, one patent in United Kingdom, one Australian patent, one Japanese patent, one Russian patent, one Chinese patent, one Hong Kong patent and three pending patent applications in other jurisdictions, including Canada, South Korea and Brazil. We expect that any patents that issue in this second family will expire in March 2034.

- For our omburtamab patent portfolio, we have an exclusive license from MSK to MSK's rights in two patent families. The first family consists of patents and patent applications with composition of matter claims covering antibodies produced by a distinct hybridoma cell line, antibodies comprising specific sequences, polypeptides comprising specific sequences, and process claims covering a method of inhibiting the growth of tumor cells, a method for imaging a tumor in a subject and a method for treating a mammalian subject, and includes eight U.S. patents, one German patent, one Spanish patent, one French patent, one patent in United Kingdom, one Italian patent and two Canadian patents. We expect that any patents that issue in this first family will expire between October 2021 and January 2026. A core U.S. patent in this family is expected to expire on January 19, 2026 and core patents in Germany, Spain, France, United Kingdom and Italy in this family are expected to expire on March 6, 2023. The second family consists of patents and patent applications with process claims covering a method of improving the prognosis or prolonging the survival of a subject bearing a tumor, and includes one Chinese patent, one Indian patent, one Canadian patent, and one pending patent application in Europe. We expect that any patents that issue in this second family will expire in March 2028. Core patents in Canada, China, and India in this family are expected to expire on March 24, 2028.

- For our huB7-H3 patent portfolio, we have an exclusive license from MSK to MSK's rights in one patent family consisting of a patent and patent applications with composition of matter claims covering antibody agents that bind specifically to protein 2Ig-B7H3 or 4Ig-B7H3, and includes two patents in the United States and Europe and 11 pending patent applications in other jurisdictions, including Canada, Australia, New Zealand, China, Japan, South Korea, Eurasia, India, Brazil, South Africa, and Hong Kong. We expect that any patents that issue in this family will expire in August 2035. In addition an international patent application has been filed, with MSK and the Company as applicants, claiming a method for treating a central nerve system (CNS) cancer using huB7H3, as well as ^{177}Lu-DTPA-8H9 conjugates. Request for entry into the national phase has been filed in the United States, Canada, Europe, Australia, New Zealand,

Japan, China, South Korea, India, Brasil, Eurasian, Russia and Hong Kong. We expect that any patent that issue in this family will expire in May 2038.

- Our Multimerization Technology patent portfolio, which inter alia relates to nivatrotamab, includes one patent family under which we have a partly exclusive license to MSK's rights in the patent application. The license is exclusive for MSK's rights in the patents rights of this family that claim products, such as bispecific antibodies which are also claimed by other patent rights licensed from MSK, and non-exclusive for patents rights of this family that claim a product that is not claimed by another patent right licensed from MSK. This family consists of patents and patent applications with composition of matter claims covering bispecific binding agents comprised of two fusion proteins, and includes one U.S. patent, one Japanese patent, one Australian patent, one German patent, one French patent one South Korean patent, one Russian patent and one patent in United Kingdom, one pending patent application in the United States and four pending patent applications in other jurisdictions, including Canada, China, Hong Kong and Brazil. In Hong Kong, request for registration and grant has been filed. We expect that any patents that issue in this family will expire in March 2034. A core U.S. patent in this family is expected to expire on March 25, 2034.

- Our CD33 antibody patent portfolio, which includes one patent family under which we have an exclusive license from MSK to MSK's rights in the patent application. This family consists of one pending patent application in the United States, and eleven pending patent applications in other jurisdictions, including Europe, Australia, Brasil, Canada, China, Eurasia, India, Japan, South Korea, New Zealand, Russia and Hong Kong relating to anti Siglec-3 (CD33) antibodies generated from a specific principal investigator's laboratory at MSK. We expect that any patents that issue in this family will expire in April 2038.

- Our GD2-GD3 Vaccine patent portfolio, which inter alia relates to a vaccine for stimulation or enhacing production of an antibody which recognized a specific ganglioside, includes five U.S. patents, with expected expiration in 2022.

- For our DOTA-PRIT or SADA patent portfolio, we have an exclusive license from MSK and MIT to MSK's and MIT's rights in the field of Radioimmunotherapy for the diagnosis and treatment of cancer. The license gives access to seven patent families owned by MSK and one patent family owned by MIT. The first patent family covers bispecific antibodies capable of binding A33 and DOTA, and use thereof for the treatment of cancer. This first patent family consists of granted patents in the United Kingdom, France, Germany, Spain, Italy, the Netherlands and Hong Kong and pending patent applications in the United States, China, Canada, Israel, Japan and Australia. We expect that any patents granted in this first family will expire in February 2036. The second family covers specific bispecific antibodies binding A33 and DOTA, and the use thereof for the treatment of cancer. This second family consists of pending applications in Australia, Canada, China, Europe, Hong Kong, Israel, Japan, India, South Korea, New Zealand, Eurasia and the United States. We expect that any patents granted in this second family will expire in September 2038. The third family covers Herceptin conjugated for Pretargeted Radioimmunotherapy and application as a theranostic product. This third patent family consists of pending applications in Australia, Canada, Brazil, China, Hong Kong, Japan, Eurasia, Europe, India, South Korea, New Zealand and the United States. We expect that any patents granted in this third family will expire in March 2039. The fourth patent family covers a multimeric antibody for two step targeting (SADA). This fourth patent family consists of patent applications in Australia, Canada, Europe and the United States. We expect that any patents granted in this fourth family will expire in May 2038. The fifth patent family covers the use of small molecule haptens for pretargeted radioimmunotherapy (PRIT) using DOTA and bispecific antibodies. This fifth patent family consists of patent applications in Australia, Canada, China, Europe, Hong Kong, Japan and the United States. We expect that any patents granted in this fifth family will expire in July 2038. The sixth patent family covers new clearing agents for DOTA-PRIT. This sixth patent family consists of an International patent application (PCT) and is expected to be filed in Australia, Canada, New Zealand, Brazil, China, India, Hong Kong, Japan, South Korea, Eurasia, Europe and the United States. We expect that any patents granted in this family will expire in July 2039. The seventh patent family covers PET-based methods for individualizing tumor targeting of antibodies. This seventh patent family consists

of granted patents in the United Kingdom, France, Germany, Spain, Italy, the Netherlands and the United States. We expect that patents granted in this seventh family will expire in May 2036. The eighth patent family, owned by MIT, covers bispecific antibodies binding DOTA. This eight patent family consists of granted patents in Belgium, France, Germany, Ireland, Italy, Spain, Switzerland, the United Kingdom and the United States. We expect that patents in this eighth family expire in February 2030.

The term of individual patents depends upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, the patent term is generally 20 years from the earliest claimed filing date of a non-provisional patent application in the applicable country. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the United States Patent and Trademark Office, or USPTO, in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over a commonly owned patent or a patent naming a common inventor and having an earlier expiration date. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration date of a U.S. patent as partial compensation for the length of time the drug is under regulatory review while the patent is in force. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. We cannot provide any assurance that any patent term extension with respect to any U.S. patent will be obtained and, even if obtained, what the duration of such extension may be.

Similar provisions are available in the European Union and certain other non-U.S. jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our product candidates receive approval by the FDA or non-U.S. regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical trials for each drug and other factors. The expiration dates referred to above are without regard to potential patent term extension or other market exclusivity that may be available to us. However, we cannot provide any assurance that any such patent term extension of a non-U.S. patent will be obtained and, even if obtained, the duration of such extension.

As for the immunotherapy products and processes we develop and commercialize, in the normal course of business, we intend to pursue, when possible, composition, method of use, dosing and formulation patent protection. We may also pursue patent protection with respect to manufacturing and drug development processes and technology.

Individual patents extend for varying periods of time, depending upon the date of filing of the patent application, the date of patent issuance, and the legal term of patents in the countries in which they are obtained. Generally, patents issued for applications filed in the United States are effective for 20 years from the earliest effective filing date. In addition, in certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of the FDA regulatory review period. The restoration period cannot be longer than five years and the total patent term, including the restoration period, must not exceed 14 years following FDA approval. The duration of patents outside of the United States varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date. Generally, as noted above, our in-licensed issued patents in all jurisdictions will expire on dates ranging from 2021 to 2035. If patents are issued on our pending patent applications, the resulting patents are projected to expire on dates ranging from 2021 to 2039. However, the actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country, and the validity and enforceability of the patent.

Trademarks

We have obtained USPTO trademark registration of the "Y-mAbs" mark, USPTO and EU trademark registration as well as registration in other jurisdictions of DANYELZA and certain other trademarks that we intend to use to commercialize our product candidates. We currently rely on our registered and unregistered trademarks, trade names and service marks, as well as our domain names and logos, as appropriate, to market our brands and to build and maintain brand recognition. We are seeking to register and will continue to seek to register and renew, or secure by

contract where appropriate, trademarks, trade names and service marks as they are developed and used, and reserve, register and renew domain names as appropriate.

Trade Secrets

We may also rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets are difficult to protect. We seek to protect our technology and product candidates, in part, by entering into confidentiality agreements with those who have access to our confidential information, including our employees, contractors, consultants, collaborators, and advisors. We also seek to preserve the integrity and confidentiality of our proprietary technology and processes by maintaining physical security of our premises and physical and electronic security of our information technology systems. Although we have confidence in these individuals, organizations, and systems, agreements or security measures may be breached and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or may be independently discovered by competitors. To the extent that our employees, contractors, consultants, collaborators, and advisors use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions. For this and more comprehensive risks related to our intellectual property and proprietary technology, inventions, improvements and products, please see the section on "Risk Factors—Risks Related to Our Intellectual Property."

MSK Agreements

On August 20, 2015, we entered into the MSK License, which grants us a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics and cancer treatments. The MSK License is exclusive with respect to MSK rights in such patent rights and tangible materials within such know-how, and nonexclusive with respect to MSK's rights in such know-how and related intellectual property rights. The patents and patent applications covered by the MSK License are directed, in part, to the DANYELZA and omburtamab antibody families, including humanized and chimeric antibodies, as well as MSK's rights in BsAbs, compositions, and their respective use for immunotherapy. Upon entering into the MSK License in 2015 and in exchange for the licenses thereunder, we paid to MSK an upfront payment of $500,000, issued 1,428,500 shares of our common stock to MSK and agreed to provide certain anti-dilution rights to MSK as further described below. In addition, we are required to pay to MSK certain royalty and milestone payments.

The MSK License requires us to pay to MSK mid to high single-digit royalties based on annual net sales of licensed products or the performance of licensed services by us and our affiliates and sublicensees. We are required to pay annual minimum royalties of $80,000 over the royalty term, starting in 2020, which amounts are non-refundable but are creditable against royalty payments otherwise due thereunder. Total expensed minimum royalty payments under the MSK License were $1,200,000 in 2016 upon determination that the payment of such minimum royalties was probable and the amount was estimable. As of December 31, 2020, of the $1,120,000 accrued, $80,000 was recorded as short-term accrued liabilities and $1,040,000 was recorded as long-term accrued liabilities. As of December 31, 2019, of the $1,200,000 accrued, $29,000 was recorded as short-term accrued liabilities and $1,171,000 was recorded as long-term accrued liabilities. We are also obligated to pay to MSK certain clinical, regulatory and sales-based milestone payments under the MSK License, which payments become due upon achievement of the related clinical, regulatory or sales-based milestones. Certain of these clinical and regulatory milestone payments become due at the earlier of completion of the related milestone activity or the date indicated in the MSK License. Total clinical and regulatory milestones potentially due under the MSK License are $2,450,000 and $9,000,000, respectively. There are also sales-based milestones that become due should we achieve certain amounts of sales of licensed products resulting from the license arrangement with MSK, with total potential sales-based milestones potentially due of $20,000,000. As product candidates progress through clinical development, regulatory approval and commercialization, certain milestone payments will come due either as a result of the milestones having been met or the passage of time even if the milestones have not been met. We will also owe MSK mid to high single-digit royalties on commercial sales of our approved products, including an annual fixed minimum royalty of $80,000 over the royalty term starting in 2020 whether or not product sales are ever achieved. In addition, to the extent we enter into sublicense arrangements, we are required to pay to MSK a percentage of certain payments that we receive from sublicensees of the rights licensed to us by MSK, which percentage will be based upon the date we receive such payments or the achievement of certain clinical milestones. We

have entered into sublicenses and distribution agreements related to DANYELZA and omburtamab under the MSK License with Takeda, Swixx and SciClone in 2020. In 2019 and 2020, we incurred milestone,royalty, and license expenses of $75,000 and $2,203,000 under MSK license agreement.

The terms of the MSK License provide that MSK is entitled to receive 40% of the income generated from the sale of first 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. We sold the PRV received upon FDA approval of DANYELZA to United Therapeutics for $105 million. Pursuant to the agreement with MSK, we were entitled to retain 60% of the net proceeds from monetization of the PRV, and the remaining 40% was due to MSK. We received our portion of the net proceeds of from the sale of the PRV in the amount of approximately $62.0 million when the transaction was consummated in January 2021.

The MSK License will expire, on a country-by-country basis, and on a licensed-product-by-licensed-product or licensed-service-by-licensed-service basis, on the later of (i) the expiration of the last to expire of the patents and patent applications covering such licensed product or service in such country, (ii) the expiration of any market exclusivity period granted by a regulatory authority for such licensed product or service in such country, or (iii) 15 years from the first commercial sale of such licensed product or service in such country.

MSK may terminate the MSK License upon prior written notice in the event of our uncured material breach, or upon prior written notice if such breach is of a payment obligation. MSK may also terminate the MSK License upon written notice in the event of our bankruptcy or insolvency or our conviction of a felony relating to the licensed products, or if we challenge the validity or enforceability of any licensed patent right. In addition, we have the right to terminate the MSK License in its entirety at will upon prior written notice to MSK, but if we have commenced the commercialization of licensed products and/or licensed services we can only terminate at will if we cease all development and commercialization of such licensed products and/or licensed services.

Our failure to meet certain conditions under the MSK License could cause the related license to such licensed product to be canceled and could result in termination of the MSK License by MSK.

On November 10, 2015, we entered into the Sponsored Research Agreement, or the SRA, with MSK pursuant to which we committed to provide aggregate research funding to MSK for a term of five years. The research will be conducted in accordance with a written plan and budget approved by the parties. MSK has granted us a non-exclusive, non-commercial, non-transferable, royalty-free license to use any inventions or discoveries developed by MSK within the scope of the information resulting from the project, for our internal, non-commercial research purposes. We have also been granted both a first option to negotiate an exclusive or non-exclusive commercial license to MSK’s rights in inventions developed by MSK and a first option to negotiate an exclusive license to MSK’s rights in inventions jointly developed by the parties. The SRA was amended on September 12, 2019, and will expire five years from the date of the amendment. The SRA may be terminated for convenience by either party upon prior written notice. During 2019 and 2020 we incurred research and development expenses of $1,283,000 and $1,617,000 respectively, under the SRA.

On September 20, 2016, we entered into a Master Data Services Agreement, or the MDSA, with MSK pursuant to which we committed to make certain payments to MSK annually in exchange for certain services, including transfer of clinical data and databases, regulatory files and other know-how to us by employees at MSK who are specifically assigned to assist with such services to us. The MDSA will expire upon the completion of activities set forth in each project description entered into thereunder; however we have the option to extend the term upon written notice to MSK. Either party may terminate the MDSA upon prior written notice in the event of an uncured material breach. During 2019 and 2020, we incurred expenses of $918,000 and $1,031,000, respectively, under the MDSA.

On June 21, 2017, we entered into the Investigator-Sponsored Master Clinical Trial Agreement, or the MCTA, as later amended on October 11, 2017, with MSK pursuant to which we committed to provide aggregate funding to MSK up to a certain amount for clinical studies to be conducted at MSK. Each such clinical study will be conducted in accordance with a written plan and budget and protocol approved by the parties. Under the MCTA, we and MSK have granted each other a non-exclusive, non-transferable, worldwide, royalty-free license, without right to sublicense, to use any inventions or discoveries developed by personnel of each such party, that is within the scope of the information

resulting from the relevant study, for the other party's internal, non-commercial research purposes until such Invention is commercially available. We have also been granted a first option to negotiate an exclusive or non-exclusive commercial license to MSK's rights in inventions or discoveries developed by MSK personnel under this MCTA and a first option to negotiate an exclusive license to MSK's rights in inventions or discoveries jointly developed by MSK and our personnel under this MCTA. The MCTA will continue in effect through completion of the studies, and may be terminated by either party upon prior written notice. During 2019 and 2020, we incurred research and development expenses of $3,128,000 and $1,118,000 under the MCTA.

On June 27, 2017, we entered into two separate Core Facility Service Agreements, or CFSAs, with MSK pursuant to which we committed to make certain payments to MSK in exchange for certain laboratory services over the term of the CFSAs. Either party may terminate either of these CFSAs for any reason, or for no reason, upon prior written notice. In the event of termination of either of these CFSAs, we will make full payment to MSK for all work performed on, or expenses related to the project up to the date of termination including all non-cancelable obligations following receipt from MSK of any completed or in-process deliverables in connection with the project. During 2019 and 2020, we incurred research and development expenses of $816,000 and $454,000, respectively, under the CFSAs.

On November 13, 2017, we entered into the MSK CD33 License, with MSK, which grants us a worldwide, sub-licensable license to MSK's rights in certain patent rights and intellectual property rights related to certain know-how to develop, make and commercialize licensed products and to perform services for all therapeutic and diagnostic uses in the field of cancer diagnostics in connection with certain CD33 antibodies generated in a specific principal investigator's laboratory at MSK and constructs thereof. The MSK CD33 License is exclusive with respect to such patent rights and tangible materials within such know-how, and nonexclusive with respect to MSK's rights in such know-how and related intellectual property rights. As product candidates progress through clinical development, regulatory approval and commercialization, certain milestone payments will come due either as a result of the milestones having been met or the passage of time even if the milestones have not been met. Also, we will owe MSK customary royalties on commercial sales of our approved products, if any. Total potential milestones due under the MSK CD33 License are $550,000, $500,000 and $7,500,000 for clinical, regulatory and sales based milestones, respectively. In addition, the MSK CD33 License contains minimum royalty payments that become due beginning in year 10 of $40,000 per year over the royalty term, increasing to $60,000 once a patent within the licensed rights is issued, subject to increase and creditable against any royalty payments due based on sales in the future. We are required to pay mid to high single-digit royalties on sales of licensed products. We also paid MSK approximately $1,360,000 for research services related to the intellectual property licensed under the MSK CD33 License. The research services occurred over the two-year period immediately following the date of the MSK CD33 License. Additionally, the terms of the MSK CD33 License provide that MSK is entitled to receive 25% of any income generated from the sale of any PRV or the sale of other comparable incentives provided by any non-U.S. jurisdiction.

The MSK CD33 License will expire, on a country-by-country basis, and on a licensed product-by-licensed-product or licensed-service-by-licensed-service basis, on the later of (i) the expiration of the last to expire of the patents and patent applications covering such licensed product or service in such country, (ii) the expiration of any market exclusivity period granted by a regulatory authority for such licensed product or service in such country, or (iii) 15 years from the first commercial sale of such licensed product or service in such country.

MSK may terminate the MSK CD33 License upon prior written notice in the event of our uncured material breach, or upon prior written notice if such breach is of a payment obligation. MSK may also terminate the MSK CD33 License upon written notice in the event of our bankruptcy or insolvency or our conviction of a felony relating to the licensed products, or if we challenge the validity or enforceability of any licensed patent right. In addition, we have the right to terminate the MSK CD33 License in its entirety at will upon prior written notice to MSK, but if we have commenced the commercialization of licensed products and/or licensed services we can only terminate at will if we cease all development and commercialization of such licensed products and/or licensed services.

On November 13, 2017, in connection with the MSK CD33 License, we entered into the Sponsored Research Agreement, or the CD33 SRA, with MSK pursuant to which we committed to provide aggregate research funding to MSK annually for a term of two years. The term of the CD33 SRA expired on November 13, 2019. The research was conducted in accordance with a written plan and budget approved by the parties. MSK had granted us a non-exclusive,

non-commercial, non-transferable, royalty-free license to use any inventions or discoveries developed by MSK within the scope of the information resulting from the research, for our internal, non-commercial research purposes. We had also been granted both a first option to negotiate an exclusive or non-exclusive commercial license to MSK's rights in inventions developed by MSK personnel and a first option to negotiate an exclusive license to MSK's rights in inventions jointly developed by the parties and our personnel. In 2019 and 2020, we incurred research and development expenses of $604,000 and zero under the CD33 SRA.

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

On December 2, 2019, we entered into the Settlement and Assumption and Assignment, or SAAA, of MSK License and Y-mAbs Sublicense Agreement, or the MabVax/Y-mAbs Sublicense, between us and Mabvax dated June 27, 2018, with MabVax Therapeutics Holdings, Inc. and MabVax Therapeutics, Inc., or together, MabVax, and MSK, which became effective on December 13, 2019. Pursuant to the Mabvax/Y-mAbs Sublicense, MabVax sublicensed to us certain patent rights and know-how for development and commercialization of products for the prevention or treatment of NB by means of administering a bi-valent ganglioside vaccine granted to MabVax, pursuant to an exclusive license agreement dated June 20, 2008 between MabVax and MSK, as amended, or the MabVax/MSK License Agreement.

On March 21, 2019, MabVax filed a voluntary petition for relief under Chapter 11 of the Bankruptcy Code. The essence of the transaction created by the SAAA was for us, in light of the Chapter 11 bankruptcy proceedings affecting MabVax, to preserve the MabVax/MSK License Agreement and the rights granted to us under the MabVax/Y-mAbs Sublicense and for us to create a direct relationship with MSK with respect to the rights covered under the MabVax/Y-mAbs Sublicense. Pursuant to the SAAA, MabVax agreed to assume the MabVax/Y-mAbs Sublicense and the MabVax/Y-mAbs License Agreement pursuant to Section 365 of the Bankruptcy Code and concurrently to assign both of these agreements to MSK. We remain responsible for any potential downstream payment obligations to MSK related to the GD2-GD3 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 GD2-GD3 Vaccine, then we are obligated to file with the FDA for a PRV. The SAAA stipulates that, if we are granted a PRV from the FDA covering a licensed product under the MabVax/Y-mAbs Sublicense and the PRV is subsequently sold, we will pay directly to MabVax and to MSK, respectively, a total of twenty percent of the proceeds from the sale thereof. The MabVax/MSK License Agreement will expire with effect for us, on a country-by-country basis, on the later of the expiration of (i) 10 years from the first commercial sale of the licensed product in such country or (ii) the last-to-expire valid claim covering such licensed product rights at the time of and in the country of sale.

On April 15, 2020, we entered into a license agreement, or the SADA License Agreement, with MSK and Massachusetts Institute of Technology, or MIT, that grants us an exclusive, worldwide, sublicensable license to certain patent and intellectual property rights developed by MSK and MIT 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 using SADA-BiDE (2-step Self-Assembly and DisAssembly-Bispecific DOTA-Engaging antibody system) Pre-targeted Radioimmunotherapy Platform, or the SADA Technology, a concept we also refer to as Liquid Radiation™. The patents and patent applications covered by the SADA License Agreement are directed, in part, to the SADA Technology, as well as a number of SADA constructs developed by MSK. Upon entering into the SADA License Agreement in April 2020 and in exchange for the licenses, we paid MSK and MIT a cash upfront payment and issued an aggregate of 42,900 shares of our common stock to them.

The SADA License Agreement requires us to pay to MSK and MIT 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, increasing to $60,000 once a patent has been issued, over the royalty term, commencing on the tenth anniversary of the license agreement. These amounts are non-refundable but are creditable against royalty payments otherwise due under the SADA License Agreement.

Under the SADA License, we are also obligated to pay MSK and MIT certain clinical, regulatory and sales-based milestone payments. 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 SADA License Agreement. Total clinical and regulatory milestones potentially due under the SADA License Agreement are $4,730,000 and $18,125,000, respectively. There are also sales-based milestones, totaling $23,750,000, that become due should the Company achieve certain amounts of sales of licensed products. In addition, for each of the SADA constructs generated by MSK and sold for the Company by a sublicensee, the Company may pay sales milestones in the total amount up to $60,000,000 based on the achievement of various cumulative net sales made by the sub-licensee. Finally, under the terms of the SADA License, 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.

Under the SADA License Agreement, we also committed to funding scientific research at MSK for up to $1,500,000 over the next three years. Accordingly, in October 2020, we entered into a SADA sponsored research agreement with MSK pursuant to which we agreed to fund $1,500,000 in scientific research at MSK over the next three years to related to the intellectual property licensed under the SADA License Agreement.

Government Regulation

The FDA and other regulatory authorities at federal, state, and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring, and post-approval reporting of biologics such as those we are developing. We, along with third-party contractors, will be required to navigate the various pre-clinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources.

The process required by the FDA before biologic product candidates may be marketed in the United States generally involves the following:

- completion of pre-clinical laboratory tests and animal studies performed in accordance with the FDA's GLP regulation;
- submission to the FDA of an IND, which must become effective before clinical trials may begin and must be updated annually or when significant changes are made;
- approval by an independent Institutional Review Board, or IRB, or ethics committee at each clinical site before the trial is begun;
- performance of adequate and well-controlled human clinical trials to establish the safety, purity and potency of the proposed biologic product candidate for its intended purpose;
- preparation of and submission to the FDA of a BLA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMP and to assure that the facilities, methods and controls are adequate to preserve the biological product's continued safety, purity and

potency, and of selected clinical investigations to assess compliance with Good Clinical Practices, or GCPs; and

- FDA review and approval of our BLA to permit commercial marketing of the product for particular indications for use in the United States, which must be updated annually when significant changes are made.

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies. The IND also includes results of animal and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

A clinical trial involves the administration of the investigational product to human patients under the supervision of qualified investigators in accordance with GCPs, which includes the requirement that all research patients provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the patients are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for patients or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- Phase 1—The investigational product is initially introduced into healthy human patients with the target disease or condition. In oncology, clinical Phase 1 trials are normally conducted in patients, who have been exposed to and failed/relapsed on available standard of care therapies. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.

- Phase 2—The investigational product is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.

- Phase 3—The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to

establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

- In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of our BLA.

Phase 1, Phase 2 and Phase 3 testing may not be completed successfully within a specified period, if at all, and there can be no assurance that the data collected will support FDA approval or licensure of the product. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and potency. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

BLA Submission and Review by the FDA

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, non-clinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from pertinent pre-clinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of the product, or from a number of alternative sources, including studies initiated by investigators. The submission of a BLA requires payment of a substantial User Fee to FDA, and the sponsor of an approved BLA is also subject to annual program fees. These fees are typically increased annually. A waiver of user fees may be obtained under certain limited circumstances.

Once a BLA has been submitted, the FDA's goal is to review the application within 10 months after it files the application, or, if the application relates to an unmet medical need in a serious or life-threatening indication, six months after the FDA files the application. The review process is often significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed, or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may convene an advisory committee to provide clinical insight on application review questions. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The testing and approval process requires substantial time, effort and financial resources, and each may take several years to complete. The FDA may not grant approval on a timely basis, or at all, and we may encounter difficulties or unanticipated costs in our efforts to secure necessary governmental approvals, which could delay or preclude us from marketing our products. After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the product will be produced, the FDA may issue an approval letter, or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter may request additional information or clarification.

The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing regulatory standards is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies. In addition, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

A sponsor may seek approval of its product candidate under programs designed to accelerate FDA's review and approval of new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for fast track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address an unmet medical need for the condition. For a fast track product, the FDA may consider sections of the BLA for review on a rolling basis before the complete application is submitted if relevant criteria are met. A fast track designated product candidate may also qualify for priority review, under which the FDA sets the target date for FDA action on the BLA at six months after the FDA files the application. Priority review is granted when there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of 10 months after FDA files the application. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval program, the FDA may approve a BLA on the basis of either a surrogate objective that is reasonably likely to predict clinical benefit, or on a clinical objective that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval are generally required to verify the biologic's clinical benefit in relationship to the surrogate objective or ultimate outcome in relationship to the clinical benefit. In addition, a sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the product candidate is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant objectives, such as substantial treatment effects observed early in clinical development. Sponsors may request the FDA to designate a breakthrough therapy at the time of or any time after the submission of an IND, but ideally before an end-of-Phase 2 meeting with FDA. If the FDA designates a breakthrough therapy, it may take actions appropriate to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the therapy; providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the non-clinical and clinical data necessary for approval is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and considering alternative clinical trial designs when scientifically appropriate, which may result in smaller trials or more efficient trials that require less time to complete and may minimize the number of patients exposed to a potentially less efficacious treatment. Breakthrough designation also allows the sponsor to file sections of the BLA for review on a rolling basis.

Fast Track designation, priority review and BTD do not change the standards for approval but may expedite the development or approval process.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, defined as a disease or condition with either a patient population of fewer than 200,000 individuals in the United States, or a patient population greater of than 200,000 individuals in the United States when there is no reasonable expectation that the cost of developing and making available the drug or biologic in the United States will be recovered from sales in the United States for that drug or biologic. ODD must be requested before submitting a BLA. After the FDA grants ODD, the generic identify of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

If a product that has received ODD and subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of ODD are tax credits for certain research and a waiver of the BLA application user fee.

A designated orphan drug many not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received ODD. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Rare Pediatric Disease Designation

The Rare Pediatric Disease Priority Review Voucher Program, or the PRV Program, is intended to incentivize pharmaceutical companies to develop drugs for rare pediatric diseases. A company that obtains approval of an IND or a BLA for a designated rare pediatric disease may be eligible for a PRV from the FDA, which may be redeemed to obtain priority review for a subsequent new drug application or BLA by the owner of such PRV. A PRV is fully transferable and can be sold to any company, who in turn can redeem the PRV for priority review of a marketing application in six months, compared to the standard timeframe of approximately ten months. A drug that receives a RPDD before September 30, 2024 continues to be eligible for a PRV if the drug is approved before September 30, 2026. Extension beyond these dates will require further Congressional action.

Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance. We cannot be certain that we or our present or future suppliers will be able to

comply with the cGMP regulations and other FDA regulatory requirements. If our present or future suppliers are not able to comply with these requirements, the FDA may, among other things, halt our clinical trials, require us to recall a product from distribution, or withdraw approval of the BLA.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved BLA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing. The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Pediatric Studies and Exclusivity

Under the Pediatric Research Equity Act of 2003, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time. For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of an applicant, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

The FDA Reauthorization Act of 2017 established new requirements to govern certain molecularly targeted cancer indications. Any company that submits a BLA three years after the date of enactment of that statute must submit pediatric assessments with the BLA if the biologic is intended for the treatment of an adult cancer and is directed at a molecular target that FDA determines to be substantially relevant to the growth or progression of a pediatric cancer. The investigation must be designed to yield clinically meaningful pediatric study data regarding the dosing, safety and preliminary efficacy to inform pediatric labeling for the product.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

Other Healthcare Laws and Compliance Requirements

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of drug products that are granted regulatory approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain our business and/or financial arrangements. Such restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration (including any kickback, bribe or rebate), directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, lease or order of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;

- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willingly executing, or attempting to execute, a scheme or making false statements in connection with the delivery of or payment for health care benefits, items, or services;

- IPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information on covered entities and their business associates that associates that perform certain functions or activities that involve the use or disclosure of protected health information on their behalf;

- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or collectively the ACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the U.S. Department of Health and Human Services, information related to payments and other transfers of value to physicians and teaching hospitals and information regarding ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

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

Also, the U.S. Foreign Corrupt Practices Act and similar worldwide anti-bribery laws generally prohibit companies and their intermediaries from making improper payments to foreign officials for the purpose of obtaining or retaining business. No assurance can be given that our internal control policies and procedures will protect us from reckless or negligent acts committed by our employees, future distributors, partners, collaborators or agents. Violations of these laws, or allegations of such violations, could result in fines, penalties or prosecution and have a negative impact on our business, results of operations and reputation.

Coverage and Reimbursement

Sales of pharmaceutical products depend significantly on the availability of third-party coverage and reimbursement. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. Third-party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. In addition, significant uncertainty exists as to the reimbursement status of newly approved healthcare products. We may need to conduct expensive clinical studies to demonstrate the comparative cost-effectiveness of our products. The product candidates that we develop may not be considered cost-effective. It is time consuming and expensive for us to seek coverage and reimbursement from third-party payors. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement may not be available or sufficient to allow us to sell our products on a competitive and profitable basis.

Review and Approval of Medicinal Products in the European Union

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, an applicant will need to obtain the necessary approvals by the comparable non-U.S. regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. Specifically, the process governing approval of medicinal products in the European Union generally follows the same lines as in the United States. It entails satisfactory completion of pre-clinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing authorization by these authorities before the product can be marketed and sold in the European Union.

The Clinical Trials Directive 2001/20/EC, the Directive 2005/28/EC on Good Clinical Practice, or GCP, and the related national implementing provisions of the individual EU member states, or EU Member States govern the system for the approval of clinical trials in the European Union. Under this system, an applicant must obtain prior approval from the competent national authority of the EU Member States in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial at a specific study site after the competent ethics committee has issued a favorable opinion. The clinical trial application must be accompanied by, among other documents, an investigational medicinal product dossier, or the Common Technical Document, with supporting information prescribed by Directive 2001/20/EC, Directive 2005/28/EC, where relevant the implementing national provisions of the individual EU Member States and further detailed in applicable guidance documents.

In April 2014, the new Clinical Trials Regulation, (EU) No 536/2014, or the Clinical Trials Regulation, was adopted, and is anticipated to enter into force in 2021. The Clinical Trials Regulation will be directly applicable in all the EU Member States, repealing the current Clinical Trials Directive 2001/20/EC. Conduct of all clinical trials performed in the European Union will continue to be bound by currently applicable provisions until the new Clinical Trials Regulation becomes applicable. The extent to which on-going clinical trials will be governed by the Clinical Trials Regulation will depend on when the Clinical Trials Regulation becomes applicable and on the duration of the individual clinical trial. If a clinical trial continues for more than three years from the day on which the Clinical Trials Regulation becomes applicable, the Clinical Trials Regulation will at that time begin to apply to the clinical trial.

The Clinical Trials Regulation aims to simplify and streamline the approval of clinical trials in the European Union. The main characteristics of the regulation include: a streamlined application procedure via a single entry point, the "EU portal"; a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (EU Member States concerned). Part II is assessed separately by each EU Member State concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the Clinical Trials Regulation.

To obtain a marketing authorization for a product under European Union regulatory systems, an applicant must submit an MAA either under a centralized procedure administered by the EMA, or one of the procedures administered by competent authorities in the EU Member States (decentralized procedure, national procedure or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the European Union. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the European Union, applicants have to demonstrate compliance with all measures included in an EMA-approved Pediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver or (3) a deferral for one or more of the measures included in the PIP.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all EU Member States and three of the four European Free Trade Association, or EFTA, States, Iceland, Liechtenstein and Norway. Pursuant to Regulation (EC) No 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases, including products for the treatment of cancer. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional.

Under the centralized procedure, the Committee for Medicinal Products for Human Use, or the CHMP, established at the EMA is responsible for conducting the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated evaluation might be granted by the

CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. If the CHMP accepts such request, the time limit of 210 days will be reduced to 150 days but it is possible that the CHMP can revert to the standard time limit for the centralized procedure if it considers that it is no longer appropriate to conduct an accelerated assessment. At the end of this period, the CHMP provides a scientific opinion on whether or not a marketing authorization should be granted in relation to a medicinal product. Within 15 calendar days of receipt of a final opinion from the CHMP, the European Commission must prepare a draft decision concerning an application for marketing authorization. This draft decision must take the opinion and any relevant provisions of EU law into account. Before arriving at a final decision on an application for centralized authorization of a medicinal product the European Commission must consult the Standing Committee on Medicinal Products for Human Use. The Standing Committee is composed of representatives of the EU Member States and chaired by a non-voting European Commission representative. The European Parliament also has a related "droit de regard". The European Parliament's role is to ensure that the European Commission has not exceeded its powers in deciding to grant or refuse to grant a marketing authorization.

Unlike the centralized authorization procedure, the decentralized marketing authorization procedure requires a separate application to, and leads to separate approval by, the competent authorities of each EU Member State in which the product is to be marketed. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States who, within 90 days of receipt, must decide whether to approve the assessment report and related materials. If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States.

The mutual recognition procedure similarly is based on the acceptance by the competent authorities of the EU Member States of the marketing authorization of a medicinal product by the competent authorities of other EU Member States. The holder of a national marketing authorization may submit an application to the competent authority of an EU Member State requesting that this authority recognize the marketing authorization delivered by the competent authority of another EU Member State.

In the European Union, innovative medicinal products approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity pursuant to Directive 2001/83/EC. Regulation (EC) No 726/2004 repeats this entitlement for medicinal products authorized in accordance the centralized authorization procedure. Data exclusivity prevents applicants for authorization of generics of these innovative products from referencing the innovator's data to assess a generic (abbreviated) application for a period of eight years. During an additional two-year period of market exclusivity, a generic marketing authorization application can be submitted and authorized, and the innovator's data may be referenced, but no generic medicinal product can be placed on the European Union market until the expiration of the market exclusivity. The overall 10-year period will be extended to a maximum of 11 years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity so that the innovator gains the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, pre-clinical tests and clinical trials.

A marketing authorization has an initial validity for five years in principle. The marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the EU Member State. To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide, on justified grounds relating to pharmacovigilance, to proceed with one further five year period of marketing authorization. Once subsequently definitively renewed, the marketing authorization shall be valid for an unlimited

period. Any authorization which is not followed by the actual placing of the medicinal product on the European Union market (in case of centralized procedure) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a drug can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union when the application is made, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that the marketing of the drug in the European Union would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Once authorized, orphan medicinal products are entitled to 10 years of market exclusivity in all EU Member States and in addition a range of other benefits during the development and regulatory review process including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure covering all member countries and a reduction or elimination of registration and marketing authorization fees. However, marketing authorization may be granted to a similar medicinal product with the same orphan indication during the 10 year period with the consent of the marketing authorization holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity.

In case an authorization for a medicinal product in the European Union is obtained, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include:

- Compliance with the European Union’s stringent pharmacovigilance or safety reporting rules must be ensured. These rules can impose post-authorization studies and additional monitoring obligations.

- The manufacturing of authorized medicinal products, for which a separate manufacturer’s license is mandatory, must also be conducted in strict compliance with the applicable European Union laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice. These requirements include compliance with European Union cGMP standards when manufacturing medicinal products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the European Union with the intention to import the active pharmaceutical ingredients into the European Union.

- The marketing and promotion of authorized drugs, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the European Union notably under Directive 2001/83EC, as amended, and EU Member State laws.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from EU directives and regulations, the United Kingdom’s withdrawal from the European Union, or Brexit, could materially impact the future regulatory regime which applies to products and the approval of product candidates in the United Kingdom.

Healthcare Reform

A primary trend in the United States healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.

In March 2010, the U.S. Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for drug products under government health care programs. Among the provisions of the ACA of importance to our potential product candidates are:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to two percent (2%) per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken.

Since its enactment, there have been numerous legal challenges and Congressional actions to repeal and replace provisions of the ACA. We 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.

Human Capital

We believe the success of the company depends on our ability to continue to attract, retain and motivate qualified employees. We seek to meet this objective by offering competitive compensation and benefits packages in our expanding organisation, with opportunities for our employees to thrive, grow and develop in their careers. We hold our employees to high ethical performance standards and our compensation plans include, as applicable, equity and cash compensation components designed to enable us to offer competitive base pay and attractive incentive schemes.

As of December 31, 2020, we had 125 full time employees. Of these employees, 87 were employed in research and development roles, 23 were employed in commercial roles and 15 were employed within general and administration. Women represent approximately 54% of our workforce and men represent approximately 46%.

The health and safety of our employees is of utmost importance to us. We offer comprehensive benefits to protect the health of our employees and their families. In response to the COVID-19 pandemic, we largely transitioning our workforce to a remote work model, while implementing additional safety measures for employees wishing to continue on-site work where possible.

The members of our management team are employed by both our company and Y-mAbs Therapeutics A/S, our wholly owned Danish subsidiary. As our development and commercialization plans and strategies continue to develop, we intend to continue adding a number of additional managerial, operational, sales, marketing, financial, and other personnel. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Corporate Information

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

We make available, free of charge on our website, our Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and all amendments to those reports filed or furnished with the SEC pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, as soon as reasonable practicable.

Item 1A. RISK FACTORS.

Our business is subject to numerous risks. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Annual Report on Form 10-K, including our financial statements and the related notes, and in our other filings with the SEC. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In such event, the trading price of our common stock could decline and you might lose all or part of your investment.

Risks Related to Our Financial Condition and Need for Additional Capital

We have a limited operating history and have incurred significant losses since inception. Our only product approved for sale is DANYELZA, which only recently received approval and we have never generated any substantial revenue from product sales. 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 commercial-stage biopharmaceutical company with a limited operating history. Since our inception in 2015, we have incurred significant losses each year. Our net losses were $81.0 million for the year ended December 31, 2019 and $119.3 million for the year ended December 31, 2020. As of December 31, 2020 our accumulated deficit was approximately $285.2 million. We have financed our operations principally through private placements, the initial public offering of our common stock in 2018 as well as our public offering in November 2019.

To date, we have devoted substantially all our efforts to research and development of DANYELZA our only approved product and our other lead product candidate omburtamab. On November 25, 2020, DANYELZA was approved by the FDA for the treatment, in combination with GM-CSF, of pediatric patients 1 year of age and older and adult patients R/R high-risk NB, in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. While our lead product candidate omburtamab is in registration stage clinical development, no assurance can be given that we will receive regulatory approval for the sale of omburtamab 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. Our only product approved for sale is DANYELZA, which only recently received approval and we have never generated any substantial revenue from product sales. We have only begun very limited sales and shipments of DANYELZA since February 2021 and we do not anticipate generating any substantial revenue from product sales until DANYELZA has been on the market for a period of time. No assurance can be given that we will ever receive regulatory approval for any of our product candidates other than DANYELZA. Our ability to generate revenue and achieve profitability depends significantly on our success in many factors, including:

- the successful launch and commercialization of DANYELZA and our product candidates for which we may obtain regulatory approvals and marketing authorizations, either directly or with a collaborator or distributor;
- completing research regarding, and non-clinical and clinical development of, our product candidates;
- obtaining regulatory approvals, marketing authorizations and coverage and reimbursements from payors for DANYELZA and other product candidates for which we complete clinical studies;
- developing and maintaining a sustainable and scalable manufacturing process for DANYELZA and our other product candidates, including establishing and maintaining commercially viable supply relationships with third parties including Patheon/Thermo Fisher and EMD/Merck or establishing our own manufacturing capabilities and infrastructure;
- obtaining market acceptance of DANYELZA and 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, distribution 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.

We anticipate incurring research, development, clinical trial, manufacturing and marketing costs associated with commercializing even approved products such as DANYELZA. 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 more of our product candidates, such as DANYELZA in the US, 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 DANYELZA or any other 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 DANYELZA and our lead product candidates and conducting pre-clinical studies and clinical trials of our other product candidates, and identifying additional potential product candidates. We have not yet demonstrated our ability to successfully 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 DANYELZA and our other product candidates.

Our payment obligations to MSK and MIT 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. 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. These milestone payments become due upon achievement of the related clinical, regulatory or sales-based milestone set forth in the MSK License and all milestones are accrued for when they are probable and estimable. 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 the MCTA, with MSK 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 paid 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 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 Study 101. We also remain responsible for any potential downstream payment obligations to MSK related to the GD2-GD3 Vaccine. This includes our obligation to make development milestone payments totaling $1,400,000 and mid single-digit royalty payments to MSK.

In April 2020, we entered into the SADA License Agreement which requires us to pay to MSK and MIT 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, increasing to $60,000 once a patent has been issued, over the royalty term, commencing on the tenth anniversary of the SADA License. These amounts are nonrefundable but are creditable against royalty payments otherwise due under the SADA License. We are also obligated to pay to MSK and MIT certain clinical, regulatory and sales-based milestone payments under the SADA 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 SADA License Agreement. Total clinical and regulatory milestone payments potentially due under the SADA License Agreement are $4,730,000 and $18,125,000, respectively.

Additionally, we are also obligated to make sales-based milestones payments totaling $23,750,000, that become due should the Company achieve certain amounts of sales of licensed products under the SADA License. In addition, for each of the SADA constructs generated by MSK and sold for the Company by a sublicensee of the Company, the Company may pay sales-based milestone payments in the total amount of $60,000,000 based on the achievement of various levels of cumulative net sales by the sublicensee. Under the SADA License Agreement, we have also committed to fund scientific research at MSK under a Sponsored Research Agreement for up to $1,500,000 over the next three years.

These payments could be significant and in order to satisfy our obligations to MSK and MIT, 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 until at least such time as we can generate substantial revenue from product sales. If we fail to obtain additional funding until at least such time as we can generate substantial revenue from product sales, we may be forced to delay, reduce or eliminate our research and drug development programs or current 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 commence sales and marketing of DANYELZA and conduct clinical trials of, and seek marketing approval for our lead product candidate omburtamab and our other product candidates. We expect to incur commercialization expenses, which may be significant, related to product sales, marketing, manufacturing and distribution of DANYELZA or our product candidates 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, until at least such time as we can generate substantial revenues from sales of DANYELZA or our product candidates, if approved, 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.

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 DANYELZA or 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 DANYELZA or 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 DANYELZA and our product candidates, if approved, 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, ownership interests will be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect the rights of common stockholders. 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 commercialization of DANYELZA or other products, if approved, or 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 current or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

We may expand our resources to pursue a particular product or product candidate or indication and fail to capitalize on other products or 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 products and product candidates and on specific indications such as DANYELZA for the treatment of relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow and omburtamab for central nervous system leptomeningeal metastases from neuroblastoma. As a result, we may forgo or delay pursuit of opportunities with other products or 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. 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.

Management performed its initial assessment of the effectiveness of internal control over financial reporting. This assessment included disclosures of 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 consolidated financial statements will not be prevented or detected on a timely basis. We have undertaken and are executing the activities necessary to remediate the material weaknesses identified in our assessment.

In connection with the audit of our financial statements for the years ended December 31, 2020 and 2019, 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) design and maintain controls to 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 are taking steps to address the material weaknesses identified above and have hired three additional finance and accounting professionals in 2019 and 2020, to a total of seven professionals to help mitigate the identified material weaknesses in our internal control over financial reporting by increasing the oversight and review procedures with regard to segregation of duties, financial reporting, financial processes and procedures and internal control procedures. Of these three additional finance and accounting professionals one has SEC reporting and accounting experience, and two have experience in internal controls and financial controlling. We have implemented, designed and are testing the operating effectiveness of controls to potentially remediate the material weakness. We have designed and are testing controls to remediate the control gaps identified in our annual assessment. No assurance can be given that these or other measures will fully remediate the material weaknesses described above in a timely manner. Additionally, if the costs related to compliance are significant, our results of operations and financial condition may be materially adversely affected. 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. No assurance can be given that all of our existing material weaknesses have been identified, or that we will not in the future identify additional material weaknesses.

We are required to formally attest to the effectiveness of our internal control over financial reporting pursuant to Section 404(a) of the Sarbanes-Oxley Act and our independent registered public accounting firm are required to formally attest to the effectiveness of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act for the year ended December 31, 2020. We are no longer an “emerging growth company” as of December 31, 2020 as the market value of our common stock held by non-affiliates exceeded $700.0 million as of June 30, 2020. Our remediation efforts may not enable us to avoid material weaknesses in our internal control over financial reporting in the future. The adverse report on the effectiveness of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act for the year ended December 31, 2020, by our independent registered public accounting firm could have a material adverse impact on our company and financial statements and 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.

If we are unsuccessful in building an appropriate accounting infrastructure, we may not be able to prepare and disclose, in a timely manner, our financial statements and other required disclosures, or comply with existing or new reporting requirements. Any failure to report our financial results on an accurate and timely basis could result in sanctions, lawsuits, delisting of our shares from the NASDAQ Global Select Market or other adverse consequences that would materially harm our business. If we cannot provide reliable financial reports or prevent fraud, or error, 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 only approved product DANYELZA, 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 DANYELZA or one or more of our product candidates, which might require additional clinical testing before we can seek regulatory approval and begin commercial sales.

Our only approved product DANYELZA, our product candidates and related technologies represent novel approaches to cancer treatment generally. Developing and commercializing these products therefore subjects us to a number of challenges. To date we have not generated any substantial revenues from sales of DANYELZA which is currently our only approved product. We may never be able to develop another marketable product. Our ability to generate product revenue is highly dependent on our ability to successfully commercialize DANYELZA and to obtain additional regulatory approvals of and successfully commercialize additional product candidates including in particular omburtamab. This 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. We cannot be certain that any other 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 in development 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.

DANYELZA and our lead product candidate omburtamab are our most advanced product and product candidate. Because our other product candidates are based on similar technology, if DANYELZA or omburtamab 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.

We have limited experience operating as a commercial company and the marketing and sale of DANYELZA or any future approved products may be unsuccessful or less successful than anticipated.

While we are initiating the commercial launch of DANYELZA in the United States, we have limited experience as a commercial company and there is limited information about our ability to successfully overcome many of the risks and uncertainties encountered by companies commercializing drugs in the biopharmaceutical industry. To execute our business plan, in addition to successfully marketing and selling DANYELZA, we will need to successfully:

- establish and maintain our relationships with healthcare providers who will be treating the patients who may receive our products and any future products;
- obtain adequate pricing and reimbursement for DANYELZA and any future products;
- gain regulatory authorization for the development and commercialization of our product candidates;
- develop and maintain successful strategic alliances;
- accurately forecast demand for our products and scale manufacturing to meet that demand;
- manage our spending as costs and expenses increase due to clinical trials, marketing approvals, and commercialization; and
- maintain and grow our relationship with MSK as a user of DANYELZA and any future products.

If we are unsuccessful in accomplishing these objectives, we may not be able to successfully develop product candidates, commercialize DANYELZA or any future products, raise capital, expand our business, or continue our operations.

The commercial success of DANYELZA and of any future approved products, will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

The commercial success of DANYELZA, and of any future approved products, will depend in part on market acceptance by physicians, patients, third-party payors, and others in the medical community. For example, current cancer treatments like surgery, chemotherapy or radiation therapy are well-established in the medical community, and doctors may continue to rely on these treatments. If DANYELZA or any future approved products 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 DANYELZA, and of any future product, 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 have only recently established our marketing and sales organization and have only limited experience in marketing and sale of biopharmaceutical products. We may not be successful in commercializing DANYELZA or any future approved product unless we are able to maintain and expand our sales and marketing capabilities or enter into agreements with third parties to sell and market such approved products.

We have only recently established our sales and marketing organization and have only limited experience in marketing and sale of biopharmaceutical products. We began small shipments of DANYELZA in February 2021. Other than our commercialization partnerships for DANYELZA and omburtamab covering certain territories outside the US with SciClone Pharmaceuticals International Ltd, Takeda Israel and Swixx Biopharma AG, we are not currently a party to any strategic collaboration that provides us with access to a collaborator's resources in selling or marketing drugs. To achieve commercial success for any future approved products we must successfully maintain and expand our sales and marketing organization or outsource these functions to strategic collaborators and other third parties. We have built our own focused, specialized sales and marketing organization in the United States. We continue to explore selectively establishing partnerships in markets outside the United States to support the commercialization of our product candidates for which we obtain marketing approval and that can be commercialized with such capabilities, and we are currently initiating the process of building our own sales capabilities in Europe, however, no assurance can be given that we will be successful in our efforts.

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;
- our inability to raise financing necessary to maintain and grow our commercialization infrastructure;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe DANYELZA or any future approved products, in particular in light of current reduced in-person access to medical institutions and personnel and other significant disruptions to the healthcare system and community due to COVID-19;
- 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.

Our revenues from the sale of drugs or the profitability of these revenues to us are likely to be lower from arrangements that we enter into with third parties to perform sales and marketing service (such as with SciClone Pharmaceuticals International Ltd, Takeda Israel and Swixx Biopharma AG) than if we were to market and sell any drugs that we develop ourselves. In addition, we may not be successful in entering additional 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 have limited 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 maintain and expand our sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we might not be successful in commercializing DANYELZA or any of our product candidates for which we receive marketing approval, if any. In the event that we are unable to effectively deploy our sales organization or distribution strategy on a timely and efficient basis, if at all, the commercialization of DANYELZA or our product candidates, if approved, could be delayed which would negatively impact our ability to generate product revenues.

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 actual and 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 DANYELZA or our product candidates or may develop proprietary

technologies or secure patent protection that we may need for the commercialization of DANYELZA and development of our product candidates and related technologies.

Specifically, MacroGenics, Inc. and Daiichi Sankyo Co. are developing antibodies against B7-H3. United Therapeutics Corporation, or United Therapeutics, has commercialized Unituxin (dinutuximab), an antibody against GD2, in the United States and has announced plans to seek a label expansion for Unituxin in combination with irinotecan and temozolomide for the treatment of pediatric patients with R/R NB. During the third quarter of 2020, United Therapeutics discontinued its efforts to investigate Unituxin's potential activity against adult cancerous tumors, and its efforts to develop a humanized version of Unituxin. In addition, DANYELZA 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. 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. EUSA has previously announced plans to file for registration of dinutuximab beta in the United States in 2020 in R/R NB. In January 2020, EUSA and BeiGene Ltd., or BeiGene, announced an exclusive collaboration to commercialize Qarziba® in mainland China and in November 2020 EUSA and BeiGene announced that the BLA for QARZIBA® (dinutuximab beta) was accepted by the China National Medical Products Administration and granted priority review.

We may not be the first to market even with respect to our approved products such as DANYELZA and that may affect the price or demand for DANYELZA and 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 products. We may not be able to implement our business plan if the acceptance of our products is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our products, or if physicians switch to other new drug or biologic products or choose to reserve our products 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.

The market opportunities for DANYELZA and our product candidates, if approved, 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. Also, the market opportunity for DANYELZA and our product candidates, if approved, may be smaller than we expect.

Our current target 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 DANYELZA and product candidates, which are derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, or market research. The total addressable market opportunity for DANYELZA and any other product candidates we may produce, if approved, will ultimately depend upon, among other things, the diagnosis criteria included in the final label for our current and future products for sale for these indications, acceptance by the medical community and patient access, drug pricing, and reimbursement. The number of patients in our targeted commercial markets and elsewhere may turn out to be lower than expected, possibly materially, patients may not be otherwise amenable to treatment with our drug, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

Even if we obtain significant market share for DANYELZA or our product candidates, if approved, because the potential target populations are small, we may never achieve profitability without obtaining regulatory approval for additional and broader indications, including use of DANYELZA or our product candidates, if approved, for front-line and third-line therapy.

DANYELZA is approved only as second line treatment for patients with relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow, and we expect to initially seek approval of our product candidate omburtamab also as second-line therapy for patients who have relapsed from systemic disease. Even if we would seek approval as front-line or third-line therapy for DANYELZA, omburtamab or another product candidate there is no

guarantee that they would be approved. In addition, we may have to conduct additional clinical trials prior to gaining approval for front-line or third-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.

DANYELZA or any current or future 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 or cause regulatory authorities to require labeling statements, such as boxed warnings. Even after approval, if 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 DANYELZA or any current or future 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 products or product candidates could cause us or regulatory authorities to interrupt, delay, or halt clinical trials.

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 products and product candidates to understand their side effect profiles, both for our planned clinical trials and upon any commercialization of any product candidates when approved such as DANYELZA. Inadequate training in recognizing or managing the potential side effects of our products or 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.

Undesirable side effects caused by DANYELZA or any other product or product candidate could limit the commercial profile of such product or product candidate or result in significant negative consequences such as a more restrictive label or other limitations or restrictions.

In clinical studies, DANYELZA has been shown to cause serious infusion reactions including anaphylaxis, cardiac arrest, bronchospasm, stridor, and hypotension. The most common adverse events were mainly mild and moderate and included infusion-related reaction, pain, tachycardia, vomiting, cough, nausea, diarrhea, decreased appetite, hypertension, fatigue, erythema multiforme, peripheral neuropathy, urticaria, pyrexia, headache, edema, anxiety, localized edema and irritability. DANYELZA has been approved with a boxed warning for serious infusion reactions and neurotoxicity.

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, such as for DANYELZA in the US, a product candidate 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 (such as for DANYELZA) narrow the indications for use or require additional warnings in the labeling, such as a boxed 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 DANYELZA or a 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. We have received clinical holds on our IND applications for certain of our product candidates in the past and there is no assurance that we will not be subject to additional clinical holds in the future, which may ultimately delay or otherwise adversely affect the clinical development of our product candidates. We have initiated Study 101 and such study will form the primary basis for our planned resubmission of the BLA for omburtamab to establish comparability of study population and pharmacokinetics analysis with Study 03-133 and to satisfy the confirmatory study and post-marketing requirements by the FDA. If the results of this study fail to demonstrate comparability to the satisfaction of the FDA and other comparable regulatory authorities, this may lead to a delay in the approval process for omburtamab.

Further, our product candidates may not be approved even if they achieve their primary endpoints in Phase 3 clinical 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.

Other than DANYELZA, the product candidates and related technologies we have licensed have not yet led, and may never lead, to approved products. Further, our only approved product DANYELZA was just recently approved and launched in the United States and hence its commercial potential cannot be judged with accuracy at this point in time. 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. As for DANYELZA, which has been approved by the FDA for the United States market, even if we receive approval to market our product candidates from the FDA, the EMA, or other regulatory bodies, whether for the treatment of cancers or other diseases, no assurance can be given you that any such product candidates will be successfully commercialized, widely accepted in the marketplace or more effective than other commercially available alternatives.

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, including the MSK License, the MSK CD33 License, the SADA License Agreement, 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. In addition, we anticipate that MSK, due to patients obtaining treatment at the institution, may become a major source for the distribution and administration of DANYELZA. Any disruption of our relationship with MSK could have a material adverse effect on our business, results of operations and financial condition. In addition, 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 or because the commercial potential is difficult to predict.

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

- such third parties or any current or 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 current or potential future collaborators may not pursue development and commercialization of our products or 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 current or 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 current or 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 products or product candidates;

- such third party or any current tor 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 current or 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 current or potential future collaborator that cause the delay or termination of the research, development or commercialization of our products or product candidates, or that result in costly litigation or arbitration that diverts management attention and resources;

- such third parties or any current or 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 current or 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 or product candidate; and

- such third parties or any current or 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 collaborations 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 products or product candidates could delay the development and commercialization of our products or 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 beta 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 normal business operations may, directly or indirectly, be adversely impacted by the ongoing global COVID-19 pandemic. COVID-19 and future outbreak of any highly infectious or contagious diseases, could materially and adversely affect our operations and have a material impact on our financial position. Further, the spread of the COVID-19 outbreak has caused business continuity issues of an as yet unknown magnitude and duration.

The COVID-19 pandemic, and preventative measures taken to contain or mitigate this pandemic have caused, and are continuing to cause, business slowdowns or shutdowns in various regions around the world and disruption in the global supply chain and business operations. National, state and local governments in affected regions have implemented and may continue to implement safety precautions, including quarantines, border closures, increased border controls, travel restrictions, shelter in place orders and shutdowns, resulting in business closures, work stoppages, slowdowns and delays, cancellation of events and other measures. These measures may disrupt normal business operations both in and outside of affected areas and may have significant negative impacts on businesses and financial markets worldwide. We continue to monitor our operations and applicable government recommendations, and we have made modifications to our normal operations because of the COVID-19 pandemic, including limiting travel and working from home and also implemented enhanced travel-safe policies for our employees' travel to our clinical sites. Prolonged remote working arrangements could impact employees' productivity and morale, strain our technology resources and introduce operational risks. Operating requirements may continually change due to the COVID-19 pandemic and we may experience unpredictability in our expenses, employee productivity and employee work culture. Additionally, the risk of cyber-attacks or other privacy or data security incidents may be heightened as a result of our moving increasingly towards a remote working environment, which may be less secure and more susceptible to security breaches.

The COVID-19 pandemic could affect the health and availability of our workforce as well as those of the third parties we rely on. If members of our management and other key personnel in critical functions across our organization are unable to perform their duties or have limited availability due to the COVID-19 pandemic, we may not be able to execute on our business strategy and/or our operations may be negatively impacted.

Our business, operations and clinical development timelines and plans have been and could continue to be adversely affected by COVID-19, and could be adversely impacted by other health epidemics in regions where we have concentrations of clinical trial sites or other business operations, and could cause significant disruption in the operations of CROs, CMOs and other third parties and collaborators upon whom we rely. The COVID-19 pandemic has affected multiple countries worldwide, including those where we have planned and ongoing preclinical studies and clinical trials.

Site initiation and patient enrollment could be delayed or suspended due to prioritization of hospital resources toward the COVID-19 pandemic or patients not having a desire to enroll in clinical trials due to concerns regarding COVID-19. In addition, some patients may not be able to comply with clinical trial protocols and the ability to conduct follow up visits with treated patients may be limited if patients do not want to participate in follow up visits due to concerns regarding COVID-19 or if quarantines impede patient movement or interrupt healthcare services. There may be shortages in the raw materials used in the manufacturing of our product candidates or laboratory supplies for our preclinical studies and clinical trials, in each case, because of ongoing efforts to address the outbreak. We cannot assure that the inability to collect such clinical data would not have an adverse impact on our clinical trial results. Similarly, our ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19 could be adversely impacted.

The response to the COVID-19 pandemic may redirect resources with respect to regulatory matters in a way that would adversely impact our ability to pursue marketing approvals. In addition, we may face impediments to regulatory meetings and potential approvals due to measures intended to limit in-person interactions. Furthermore, third parties, including manufacturers, medical institutions, clinical investigators, CROs and consultants with whom we conduct business, are similarly adjusting their operations and assessing their capacity in light of the COVID-19 pandemic. If these third parties continue to experience shutdowns or business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted.

Further, COVID-19 has severely impacted global economic activity and caused significant volatility and negative pressure in global financial markets. Many experts predict that the outbreak will trigger a period of global economic slowdown or a global recession. We are unable to predict the extent or nature of these impacts at this time.

COVID-19 is adversely affecting, and is expected to continue to adversely affect, our operations, and COVID-19 or another pandemic may result in material and adverse effects on our ability to successfully operate our business, including:

- our ability to successfully launch, commercialize, and generate revenue from DANYELZA and our product candidates, even if approved, may be adversely affected by the impact of the COVID-19 pandemic. For example, limited hospital access for non-patients, social distancing requirements, and precautionary measures due to COVID-19 have impacted the ability of our sales personnel to interact in-person with customers. In response, we have implemented a virtual launch model, which may adversely affect the ability of our sales professionals to effectively market DANYELZA and our product candidates to physicians, which may have a negative impact on our sales and our market penetration. In addition, in the United States we plan to utilize various programs to help patients afford our products, including patient assistance programs for eligible patients. Market disruption and rising unemployment caused by the COVID-19 pandemic may lead to increased utilization of our patient assistance programs, which could reduce revenues;

- delays in receiving approval from local regulatory authorities to initiate our planned clinical trials, including receiving any required investigational new drug applications, or INDs;

- delays or difficulties in enrolling and retaining patients in our clinical trials if patients are affected by the virus or are fearful of visiting or traveling to clinical trial sites because of the outbreak;

- risk that participants enrolled in our clinical trials will acquire COVID-19 while the clinical trial is ongoing, which could impact the results of the clinical trial, including by increasing the number of observed adverse events;

- delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;

- manufacturing disruptions;

- delays in clinical sites receiving the supplies and materials needed to conduct our clinical trials;
- delays in the transport of clinical trial materials;
- changes in local regulations as part of a response to the COVID-19 pandemic which may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- interruption of or changes in key clinical trial activities, such as clinical trial site monitoring, implementation of virtual monitoring, use of local testing labs, or home delivery of study drugs, due to limitations on travel imposed or recommended by federal or state governments, employers and others, use of new digital technologies for subject visits or interruption of clinical trial subject visits and study procedures, the occurrence of which could affect the integrity of clinical trial data;
- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees;
- delays in regulatory approvals for our product candidates due to the FDA focusing on clinical trials related to therapies and vaccines targeting COVID-19;
- refusal of the FDA or other regulatory authorities to accept data, including from clinical trials in affected geographies or failure to comply with updated FDA or other regulatory guidance and expectations related to the conduct of clinical trials during the COVID-19 pandemic;
- the destabilization of the markets and negative impacts on the healthcare system or regulatory authorities globally could negatively impact our ability to obtain approval to market, market and sell our products, including through the disruption of regulatory activities or health care activities in general;
- difficulty accessing the capital and credit markets on favorable terms, or at all, and a severe disruption and instability in the global financial markets, or deteriorations in credit and financing conditions which could affect our access to capital necessary to fund business operations;
- the potential negative impact on the health of our employees, especially if a significant number of them or any of their family members are impacted or if any of our senior leaders are impacted for an extended period of time;
- potential delays in the preparation and submission of applications for regulatory approval of our product candidates, as well as potential interruptions or delays in FDA's ability to review applications in a timely manner consistent with past practices, which may impact review and approval times;
- delays in scheduling manufacturing inspections in connection with BLA approval;
- a general decline in business activity; and
- a deterioration in our ability to ensure business continuity during a disruption.

Despite our efforts to manage and mitigate these impacts to our company, their ultimate impact also depends on factors beyond our knowledge or control, including the duration and severity of this and any other pandemic, as well as third-party actions taken to contain its spread and mitigate its public health effects, and the pace of global economic recovery following containment of the spread. In addition, while we cannot predict the impact that COVID-19 will have

on our suppliers, vendors and other business partners and each of their financial conditions, any material adverse effects on these parties could adversely impact us. The ultimate impact of this and any other pandemic on our business is highly uncertain and the continued spread of COVID-19 may have further adverse impacts on our business, operations, any pending regulatory approvals, supply chain, and financial position, and may also exacerbate other risks discussed in this this Annual Report on Form 10-K. To the extent the COVID-19 pandemic adversely affects our business, clinical trials, results of operations and financial condition, it may also have the effect of heightening many of the other risks described in this "Risk Factors" section.

The ultimate impact of the COVID-19 pandemic or a similar health epidemic is highly uncertain and subject to change.

Any significant disruption in or unauthorized access to our computer systems or those of third parties that we utilize in our operations, including those relating to cybersecurity or arising from cyber-attacks, could result in a loss or degradation of service, unauthorized disclosure of data, including member and corporate information, or theft of intellectual property which could adversely impact our business.

Our business is dependent upon the reliable performance and security of our computer systems and those of third parties that we utilize in our operations. These systems may be subject to damage or interruption from, among other things, earthquakes, adverse weather conditions, other natural disasters, terrorist attacks, state-sponsored attacks, rogue employees, power loss, telecommunications failures, and cybersecurity risks. Interruptions in these systems, or with the internet in general, could hinder our ability to operate. Service interruptions, errors in our software or the unavailability of computer systems used in our operations could diminish the overall attractiveness of our business.

Our computer systems and those of third parties we use in our operations are subject to cybersecurity threats, including cyber-attacks such as computer viruses, denial of service attacks, physical or electronic break-ins and similar disruptions. Additionally, outside parties may attempt to induce or deceive employees or users to disclose sensitive or confidential information in order to gain access to data. Any attempt by hackers to obtain our data (including patient, clinical trial and corporate information) or intellectual property, disrupt our business, or otherwise access our systems, or those of third parties we use, if successful, could harm our business, be expensive to remedy and damage our reputation. We have implemented commercially reasonable systems and processes to thwart hackers and otherwise protect our data and systems, but the techniques used to gain unauthorized access to data and software are constantly evolving, and we may be unable to anticipate or prevent unauthorized access. There is no assurance that hackers may not have a material impact on our business or systems in the future. Efforts to prevent hackers from disrupting our service or otherwise accessing our systems are expensive to develop, implement and maintain. These efforts require ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated, and such efforts may limit the functionality of or otherwise negatively impact our operations and systems. Any significant disruption to our systems could adversely affect our business and results of operation. Further, a penetration of our systems or a third-party's systems or other misappropriation or misuse of personal information could subject us to business, regulatory, litigation and reputation risk and divert internal resources to respond to such an event, which could have a negative effect on our business, financial condition and results of operations.

We utilize our own communications and computer hardware systems located either in our facilities or in that of a third-party provider. In addition, we utilize third-party "cloud" computing services in connection with our business operations. Problems faced by us or our third-party "cloud" computing or other network providers, including technological or business-related disruptions, as well as natural disasters, cybersecurity threats and regulatory interference, could adversely impact the experience of our members.

Risks related to our dependence on third parties

Third parties have sponsored most clinical trials of DANYELZA and omburtamab 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 DANYELZA and omburtamab. 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 DANYELZA and omburtamab 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 DANYELZA or omburtamab 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 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 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 GCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators, and trial sites. If we or any of these third parties fail to comply with applicable GCP 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 GCP 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 rely on third parties to manufacture DANYELZA for commercial sales and our product candidates for our ongoing and planned pre-clinical studies and clinical studies. We also expect to rely on third parties for the manufacturing process of additional product candidates and for commercial supplies of other product candidates than DANYELZA, 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 commercial or clinical-scale manufacturing and processing facility and we rely on outside vendors to manufacture supplies and process DANYELZA and product candidates for pre-clinical studies and clinical trials under the guidance of our management team. DANYELZA and omburtamab have only been manufactured or processed on a limited basis and we may not be able to continue doing so for these or 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 DANYELZA and omburtamab 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 DANYELZA and omburtamab 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 rely also on third-party manufacturers and third-party collaborators for the manufacture of DANYELZA for commercial supply and we expect this also to be the case for 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.

We are highly dependent on our current third-party drug substance manufacturer of omburtamab, EMD/Merck, since this manufacturing process uses a hybridoma cell line in a relatively small scale (200 litres) cGMP manufacturing process. Many manufacturers refuse to allow hybridoma cell lines to be used in their facilities due to the risk of contamination and the relatively small scale of the cGMP system may render a collaboration with us less attractive from a third party's commercial point of view.

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 additional 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 DANYELZA and our product candidates must be approved by the FDA pursuant to inspections conducted after submittal of the 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. DANYELZA and 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 adversely affect our commercialization of approved products, such as DANYELZA, and delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply of our DANYELZA and omburtamab and we only currently use a different single third-party manufacturer for fill-and-finish services for DANYELZA and omburtamab. If our current contract manufacturers cannot perform as agreed, we may be required to replace those manufacturers and we may incur added costs and delays in identifying and qualifying any such replacement. We have been informed that the FDA plans to visit and inspect the site of EMD Millipore Corporation (now part of the Merck KgaA group of companies), or EMD/Merck in Merck KgA site in Martillac, France, where the drug substance is manufactured. The COVID-19 pandemic and travel restrictions may impact the timing of such inspection, even though it is not expected to take place until the second half of 2021. We estimate that the FDA inspection may occur in the second half of 2021 assuming the FDA accepts the omburtamab BLA filing. However, if the FDA is not able to timely conduct an inspection for any reason, including due to COVID-19 travel restrictions or otherwise, there may be adverse consequences to the approval process, and we may not obtain BLA approval on a timely basis or at all. Delays in the approval process or our inability to obtain approval for any reason for omburtamab or any other product candidate would have a material adverse effect upon our business, results of operations and financial condition. The FDA may also decide to inspect the fill and finish site at Patheon/Thermo Fisher in Ferentino, Italy, which may cause similar risks.

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 the 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 high-risk NB and relapsed osteosarcoma using DANYELZA. We are also conducting clinical trials at MSK for CNS/LM from NB, DIPG and DSRCT for our omburtamab product candidate and GD2 positive tumors for our nivatrotamab product candidate. Under the terms of the MCTA, 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, the MSK CD33 License and the SADA License Agreement. 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 DANYELZA and our other 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, the SADA License Agreement or its other agreements with us, commercialization of any approved product, such as DANYELZA, or 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.

DANYELZA and our product candidates are biologics and the manufacture of DANYELZA and 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.

DANYELZA and 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 of DANYELZA and our product candidates 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. No assurance can be given that any stability failures or other issues relating to the manufacture of DANYELZA or 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.

We have entered into strategic collaborations for the development, marketing and commercialization of DANYELZA and omburtamab in certain jurisdictions and may do so also in the future for all or some of our product candidates. If those collaborations are not successful, or if we are unable to establish additional collaborations, we may have to alter or delay our development and commercialization plans.

As we further develop our lead product candidates and following potential approval, commercialize our products, we may build a commercial infrastructure with the capability to directly market it to a variety of markets and territories. In November 2020, we have entered into an exclusive license and distribution agreement for DANYELZA and omburtamab with Takeda Israel, a wholly owned subsidiary of Takeda Pharmaceutical Company Limited covering the State of Israel, West Bank and Gaza Strip. In December 2020, we entered into a distribution agreement for DANYELZA and omburtamab with Swixx BioPharma AG for the Eastern European territories Bosnia & Herzegovina, Bulgaria, Croatia, Czech Republic, Estonia, Hungary, Latvia, Lithuania, Poland, Romania, Russia, Serbia, Slovakia and Slovenia. Finally, later in December 2020, we entered into a license agreement for DANYELZA and omburtamab with SciClone Pharmaceuticals International Ltd., for Greater China, including Mainland China, Taiwan, Hong Kong and Macau. We may enter into further strategic collaborations for the development, marketing and commercialization of all

or some of our product candidates. Our current and future potential 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 any further collaborations 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. We have and will for any future collaborations 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 current and future potential collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, our current collaborators have and 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.

Our current and any future potential 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.

Our current and any future collaboration agreements, if any, 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 additional 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.

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

Market acceptance and sales of our products, if approved, such as DANYELZA, will depend on coverage and 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. To date, no third-party provider has established coverage policies or provided reimbursement for DANYELZA or any of our other product candidates and we cannot assure you that coverage and reimbursement will be available for DANYELZA or 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 coverage and reimbursement policies will not reduce the demand for, or the price paid for, our products. If coverage and reimbursement is not available or is available on a limited basis, or if the coverage and 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

In October 2020 we received a Refusal to File letter from the FDA regarding our BLA for omburtamab. We plan to resubmit the BLA. The FDA retains discretion to decide again not to file our BLA for omburtamab and may refuse to accept an accelerated approval pathway for omburtamab or our other product candidates which could have a material adverse impact on our development and approval process for these product candidates and our other product candidates.

We initiated submission of a rolling BLA for omburtamab in June 2020 under the FDA's rolling review process and completed the rolling submission in August 2020. In October 2020 we received a Refusal to File letter from the FDA regarding the BLA for omburtamab. The reason for the FDA's decision to issue the Refusal to File letter was that upon preliminary review, the FDA determined that certain parts of the Chemistry, Manufacturing and Control (CMC) Module and the Clinical Module of the BLA required further detail. Among other things, the FDA requested that detailed validation data be included in the CMC Module, that clinical study data and external control data be reanalyzed

using a propensity score adjusted analysis for important baseline characteristics, such as prior receipt of irradiation, and that further supportive evidence in the form of direct anti-tumor effect be included in the Clinical Module. We have been working closely with the FDA to resolve these issues and have a meeting with the FDA scheduled for March 26, 2021, to discuss the adequacy of the external control reanalysis and supporting data to demonstrate direct anti-tumor effect for a BLA resubmission. Assuming a positive outcome of these discussions, we expect to resubmit our BLA for omburtamab by the end of the second quarter of 2021 or the third quarter of 2021. However, there is no assurance that we will be successful in these discussions or that the FDA would accept our proposal and our data as sufficient to allow the BLA resubmission. In addition, the FDA may raise additional issues and pose questions to us that may delay the resubmission of our BLA for omburtamab, the filing of the BLA for omburtamab by the FDA, the approval process and the ultimate issuance of any Marketing Authorizations for omburtamab. 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, or at all, to prepare our BLA submission for omburtamab as planned. If we are unable to address all questions or concerns the FDA has raised or 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 submit our BLA and ultimately receive a Marketing Authorization for omburtamab. If the FDA files the BLA and we are delayed or unable to provide data in response to FDA information requests, the PDUFA date for our BLA may be extended or we may receive a Complete Response Letter, which would have a material adverse effect on our business, results of operation and financial condition.

The FDA retains discretion to decide again not to review our BLA for omburtamab. 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 intend to resubmit our BLA as a rolling BLA submission. However, the FDA could refuse to accept a rolling BLA submission, which could result in a delay in obtaining a Marketing Authorization, if at all. In addition, while we are currently pursuing a full approval for our BLA for omburtamab, in the event the FDA finds our data insufficient to support such full approval, it is possible that the FDA would consider whether our clinical data is sufficient for a potential accelerated approval. However, the FDA may not find our data sufficient to support either a full approval or an accelerated approval. Even if the FDA were to find our clinical data sufficient to support an accelerated approval, we would need to conduct a post-marketing confirmatory study to confirm the clinical benefit of omburtamab. The FDA may also impose other conditions as a result of any accelerated or full approval which we may not be able to satisfy. Any delay or inability to obtain approval for our BLA for omburtamab would materially adversely affect our ability to generate revenue from commercialization of omburtamab, 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. We can provide no assurance that the FDA will agree with our proposal or that we will be able to refile the BLA submission on time or at all or that we will ultimately be able to obtain FDA approval for omburtamab.

In addition, as part of the FDA approval process, the FDA will require an inspection of the manufacturing facilities for omburtamab. If the FDA is unable to conclude that these manufacturing facilities are in substantial compliance with cGMP, or if the FDA is not able to timely conduct an inspection for any reason including due to COVID-19 travel restrictions or otherwise, there may be adverse consequences to the approval process, and we may not obtain BLA approval on a timely basis or at all. We estimate that the FDA inspection may occur in the second half of 2021 if assuming the FDA accepts the omburtamab BLA filing, however, if the FDA is not able to timely conduct an inspection for any reason including due to COVID-19 travel restrictions or otherwise, there may be adverse consequences to the approval process, and we may not obtain BLA approval on a timely basis or at all. Delays in the approval process or our inability to obtain approval for any reason for omburtamab or any other product candidate would have a material adverse effect upon our business, results of operations and financial condition. Delays in the approval process or our inability to obtain approval for any reason for omburtamab or any other drug candidate would have a material adverse effect upon our business, results of operations and financial condition.

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. To date, we have only obtained regulatory approval to market DANYELZA in the United States for relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow. We cannot predict when or if, and in which other territories, we, or any of our potential future collaborators, will obtain marketing approval to commercialize a product or 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 as we did for DANYELZA for relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow. We intend to conduct additional clinical trials in the United States and Europe. We intend to discuss with the FDA and EMA submission of BLAs for respective approval of DANYELZA and omburtamab 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. The FDA may not agree with our accelerated approval strategy with respect to omburtamab. The FDA may ultimately require one or multiple Phase 3 clinical trials prior to approval of omburtamab or other product candidates.

We have some, but only limited, experience in completing a submission of a BLA to the FDA, or similar approval submissions 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 GCPs, 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;
- manufacturing qualified materials under cGMPs for use in clinical trials;
- impact of the COVID-19 pandemic; or
- inspection of clinical trial sites and manufacturing facilities by regulatory authorities.

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 DANYELZA and 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. Also, the market opportunity for DANYELZA and our product candidates may be smaller than we expect." 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, including omburtamab, 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 omburtamab or any of our other product candidates, which would significantly harm our business, results of operations and prospects. In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted drug application may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional pre-clinical studies, clinical trials, toxicology or other in vivo or in vitro data to support the initiation of other studies and testing. In addition, varying interpretations of the data obtained from pre-clinical studies and clinical trials could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any collaborators we may have in the future, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved drug not commercially viable.

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

The European Medicines Agency, or the EMA, or comparable foreign regulatory authorities, may disagree with our regulatory plans, including our plans to seek conditional marketing authorization, and we may fail to obtain regulatory approval of our DANYELZA or our product candidates, which would prevent DANYELZA or our product candidates from being marketed abroad. Any approval we are granted for our product candidates in the United States, such as the approval of DANYELZA, 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, such as the approval of DANYELZA for relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow, 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.

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 Breakthrough Therapy Designation, or BTD, for one or more of our 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.

BTD 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.

In June 2017, ^{131}I-omburtamab received BTD for the treatment of pediatric patients with R/R NB who have CNS/LM from NB. 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. BTD does not change the standards for product approval.

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 or maintain Orphan Drug Designation, or ODD, or Rare Pediatric Disease Designation, or RPDD.

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 September 2020, the FDA granted ODD and RPDD to nivatrotamab for the treatment of neuroblastoma. In November 2018, the European Commission granted OMPD for naxitamab for the treatment of NB. In February 2017, the European Commission granted orphan medicinal product designation, or OMPD, to omburtamab for the treatment of NB. In August 2016, the FDA granted ODD to ^{131}I-omburtamab for the treatment of NB. In 2013, the FDA granted ODD to DANYELZA 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 may be 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.

The Rare Pediatric Disease Priority Review Voucher Program, or PRV Program, is intended to incentivize pharmaceutical sponsors to develop drugs for rare pediatric diseases. A sponsor who obtains approval of a BLA for a rare pediatric disease may be eligible for a 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.

In conjunction with the approval by the FDA of DANYELZA, for the treatment of refractory/relapsed high-risk neuroblastoma, we received a PRV which we subsequently sold to United Therapeutics Corporation based on an agreed valuation of $105 million. Pursuant to the agreement with MSK, we were entitled to retain 60% of the net proceeds from monetization of the PRV, and the remaining 40% was due to MSK. We received our portion of the net proceeds of from the sale of the PRV in the amount of approximately $62.0 million when the transaction was consummated in January 2021. The terms of the MSK License provide that MSK is entitled to receive 40% 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. Additionally, the SAAA stipulates that, if we are granted a PRV from the FDA covering a licensed product under the MabVax/Y-mAbs Sublicense and the PRV is subsequently sold, we will pay directly to MabVax and to MSK, respectively a total of 20% of the income generated thereof in order that MabVax and MSK each receive the same amount therefrom as envisaged under the MabVax/MSK License Agreement. Finally, the terms of the SADA 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.

A drug that receives RPDD before September 30, 2024, will continue to be eligible for a PRV if the drug is approved by the FDA before September 30, 2026.

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, such as for DANYELZA in the Unites States, an approved drug and its manufacturer and marketer are subject to ongoing review and extensive regulation. The accelerated approval of DANYELZA is subject to certain postmarketing requirements and commitments, including completion of a confirmatory postmarketing trial of clinical benefit, that must be completed in order to convert the BLA to full approval and prevent withdrawal of the license by FDA. The confirmatory postmarketing clinical trial required by the FDA to verify and to further characterize the clinical benefit is our ongoing Study 201, which will enroll a minimum of 80 patients and report ORR, DOR, PFS or OS. The ORR is the primary endpoint for the study, DOR is the secondary endpoint, PFS and OS are secondary endpoints in long-term follow up. As of February 1, 2021 we have enrolled 54 patients and we anticipate completing the study no later than March 31, 2027. Other 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, in a manner 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, such as for DANYELZA, 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.

DANYELZA and 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.

DANYELZA and 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 Federal 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the Affordable Care Act, or ACA, substantially changed 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 the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

- 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.

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. 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. We 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.

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.

A May 2018 “Blueprint” to lower drug prices and reduce out of pocket costs of drugs contained 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.

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 DANYELZA and any other 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 DANYELZA or our other approved products, 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 DANYELZA and omburtamab, 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 DANYELZA or our other 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 any our future products, which would adversely affect our anticipated revenue and results of operations.

Our relationships with healthcare providers, physicians and third-party payors are 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 are subject 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 current and 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 DANYELZA and other 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 HIPAA, as amended, requires 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 then-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 most businesses, regardless of 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 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 comply with applicable healthcare laws and regulations involve substantial costs. It is possible that interpretation of healthcare laws and regulations will vary across jurisdictions, and 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. We have established internal policies and procedure to mitigate our compliance risks. However, no assurance can be given that such policies and procedures will be adequate to ensure compliance with applicable laws and regulations. Moreover, 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 DANYELZA and our product candidates, which could make it difficult for us to sell DANYELZA and our product candidates profitably.

Successful sales of DANYELZA and our product candidates, if approved, depend on the availability of adequate coverage and reimbursement from third-party payors. In addition, because DANYELZA and our product candidates represent relatively new approaches to the treatment of cancer, we cannot accurately estimate the potential revenue from DANYELZA or 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. To date, no third party provider has established coverage policies or provided reimbursement for DANYELZA or any of our other product candidates. We intend to seek reimbursement for DANYELZA but even if we obtain coverage for DANYELZA or any other 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 unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our products. Because our products and 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.

To date DANYELZA has been approved for sale in the United States only, but we intend to seek approval to market our products in both the United States as well as 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.

We may incur significant liability if enforcement authorities allege or determine that we are engaging in commercial activities or promoting DANYELZA or another product candidate in a way that violates applicable regulations.

Physicians have the discretion to prescribe drug products for uses that are not described in the product's labeling and that differ from those approved by the FDA or other applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician's choice of treatments, the FDA and other regulatory agencies regulate a manufacturer's communications regarding off-label use and prohibit off-label promotion, as well as the dissemination of false or misleading labeling or promotional materials. Manufacturers may not promote drugs for off-label uses. Accordingly, we may not promote DANYELZA in the United States for use in any indications other than relapsed/refractory high-risk neuroblastoma in bone and/or bone marrow. The FDA and other regulatory and enforcement authorities actively enforce laws and regulations prohibiting promotion of off-label uses and the promotion of products for which marketing approval has not been obtained. A company that is found to have improperly promoted off-label uses may be subject to significant liability, which may include civil and administrative remedies as well as criminal sanctions.

Notwithstanding regulations related to product promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading, and non-promotional scientific exchange concerning their products. We intend to engage in medical education activities and communicate with healthcare providers in compliance with all applicable laws and regulatory guidance.

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 products or 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. No assurance can be given 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. Similar laws in other countries, such as the U.K. Bribery Act 2010, may apply to our operations.

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.

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 products, product candidates 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 products, product candidates 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 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 MIT 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, MIT or other third parties, we may not be able to continue developing our products.

We currently in-license certain intellectual property from MSK and MIT. 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, enforceable or sufficient patents and other intellectual property rights. We have limited control over the manner in which our licensors may 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 proceedings 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 products or product candidates 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 products or 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 collaboration 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, MIT, 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 products or product candidates 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 own as well as our 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, which may also 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 products, product candidates 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 products or product candidates but that are not covered by the claims of our patents;
- the APIs in our current products or 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 products or product candidates 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 products, 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, product 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 products and product candidates. Likewise, our current owned patents and patents in-licensed from MSK relating to our proprietary technologies and our product candidates comprise patents that are expected to expire on various dates from 2021 through 2039, 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 2028 through 2039, without taking into account any possible patent term adjustments, extensions or supplementary protections. However, no assurance can be given 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 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 pertain to patents granted outside of the United States, 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 such oppositions 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 products or product candidates 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 products or product candidates 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 related to our products or product candidates, 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 products or product candidates 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 such proceedings 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 products or product candidates 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 products or product candidates 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.

Failure to secure trademark registrations could adversely affect our business.

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

Risks related to employee matters and managing growth

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

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

Risks related to our common stock

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

As of December 31, 2020, our executive officers, directors and our stockholders, which own more than 5% of our outstanding common stock beneficially own shares representing approximately 41.3% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to exert significant influence over 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 have significant influence over 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 or members of our board of directors.

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.

Effective as of December 31, 2020, we became a large accelerated filer and were no longer an emerging growth company. We are not able to take advantage of the reduced disclosure requirements applicable to emerging growth companies.

As a result of our public float (the market value of our common shares held by non-affiliates) as of June 30, 2020, we became a large accelerated filer as of December 31, 2020 and therefore no longer qualified as an "emerging growth company," as defined in the JOBS Act. Additionally, due to our public float as of June 30, 2020, we no longer qualified as a "smaller reporting company" as defined in the Exchange Act. However, we are not required to reflect the change in our smaller reporting company status, and comply with the associated increased disclosure obligations, until our quarterly report for the three-month period ended March 31, 2021.

As a large accelerated filer, we are subject to certain disclosure and compliance requirements that apply to other public companies but did not previously apply to us due to our status as an emerging growth company. These requirements include, but are not limited to:

- the requirement that our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act of 2002;
- compliance with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation;
- the requirement that we provide full and more detailed disclosures regarding executive compensation; and
- the requirement that we hold a non-binding advisory vote on executive compensation and obtain stockholder approval of any golden parachute payments not previously approved.

We expect that compliance with the additional requirements of being a large accelerated filer will increase our legal and financial compliance costs and cause management and other personnel to divert attention from operational and other business matters to devote substantial time to public company reporting requirements. In addition, if we are not able to comply with changing requirements in a timely manner, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC, or other regulatory authorities, which would require additional financial and management resources.

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, 2019. 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 43,526,254 shares of common stock outstanding as of February 22, 2021. Of these shares of our common stock, 6,900,000 shares sold in our initial public offering in 2018, 5,134,750 shares sold in our public offering in 2019 and 2,804,878 shares sold in our public offering in February 2021 are freely tradable, without restriction, in the public market. As of February 22, 2021 holders of approximately 2,744,000 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 our 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.

We may issue additional shares of our 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) is the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the DGCL or our 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 sales of our common stock by us, our insiders or other stockholders.

General risk factors

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 expect our operating results to fluctuate in future periods, which may adversely affect our stock price.

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the level of success of our commercial efforts, as well as the variable nature of our operating expenses as a result of the timing and magnitude of expenditures. In one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

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.

No assurance can be given that any clinical studies will be conducted as planned or completed on schedule, if at all. In addition, we cannot be sure that we will be able to submit INDs for any of our product candidates in the future and we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin. Moreover, even if these clinical studies 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.

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.

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, other natural or man-made disasters or business interruptions, for which we are predominantly self-insured, and other severe hazards or global health crises, such as an outbreak of Ebola or the ongoing global COVID-19 pandemic, or other actual or threatened epidemic, pandemic, outbreak and spread of a communicable disease or virus, in the countries where we operate or plan to sell our products, if approved, could adversely affect our operations and financial performance. 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 DANYELZA and 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. The ultimate extent of the impact of any epidemic, pandemic or other global health crisis on our business, financial condition and results of operations will depend on future developments which are highly uncertain and cannot be predicted, including new information that may emerge concerning the duration and severity of such epidemic, pandemic or other global health crisis, actions taken to contain or prevent their further spread and the pace of global economic recovery following containment of the spread.

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 sale of DANYELZA and clinical testing of our product candidates and will face an even greater risk if we commercialize more 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 use, 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 DANYELZA or any product candidates we develop, alone or with collaborators. The amount of clinical trial and 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.

Brexit may affect our operations.

On January 31, 2020 the United Kingdom withdrew from and ceased to be part of the European Union, commonly referred to as Brexit. Trade between European Union member states and the United Kingdom is now governed by the EU-UK Trade and Cooperation Agreement, which took effect after the Brexit transition period expired on December 31, 2020. The EU-UK Trade and Cooperation Agreement contains a number of general provisions on regulation and regulatory practice that are intended to facilitate exchange of goods and services between the European Union and the United Kingdom. However, 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. The unavoidable uncertainties and events related to Brexit could cause volatility in currency exchange rates, interest rates, and European, United Kingdom or worldwide political, regulatory, economic or market conditions and contribute to instability in political institutions, regulatory

agencies and financial markets. 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. 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.

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. As we have obtained FDA approval of DANYELZA and have begun commercializing DANYELZA in the United States, our exposure under such laws has increased significantly, and our costs associated with compliance with such laws have increased significantly and are likely to continue to increase. These laws 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.

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.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our drug, clinical development programs, and the diseases our drug and drug candidates are being developed to treat, and we are utilizing what we believe is appropriate social media in connection with our commercialization efforts for DANYELZA and we intend to do the same for our future products, if approved. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business, resulting in potential regulatory actions against us. For example, patients may use social media channels to comment on their experience in an ongoing blinded clinical study or to report an alleged adverse event, or AE. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable AE reporting obligations or we may not be able to defend our business or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our investigational products. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face regulatory actions, or incur other harm to our business.

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 22, 2018, our stock has traded at prices as low as $14.16 per share and as high as $55.22 per share through February 21, 2021. 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:

- our ability to successfully launch and commercialize DANYELZA and any other product candidates, if approved;
- the timing and results of clinical trials of any of our product candidates;
- regulatory actions with respect to our products or 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 products and 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 products, 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.

If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, investors’ views of us and, as a result, the value of our common stock.

Pursuant to Section 404 of the Sarbanes-Oxley Act, or Section 404, our independent registered public accounting firm is required to attest to the effectiveness of our internal control over financial reporting for the year ended December 31, 2020. We are no longer an “emerging growth company” as of December 31, 2020 as the market value of our common stock held by non-affiliates exceeded $700.0 million as of June 30, 2020.

Our remediation efforts may not enable us to avoid material weaknesses in our internal control over financial reporting in the future. An adverse report on the effectiveness of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act in future years, by our independent registered public accounting firm could have a material adverse impact on our company and financial statements and 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.

The rules governing the standards that must be met for management and our independent registered public accounting firm to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation. In connection with our and our independent registered public accounting firm's evaluations of our internal control over financial reporting, we may need to upgrade systems, including information technology, implement additional financial and management controls, reporting systems, and procedures, and hire additional accounting and finance staff.

Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us or our independent registered public accounting firm conducted in connection with Section 404 may reveal deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock. Internal control deficiencies could also result in a restatement of our financial results in the future. We could become subject to stockholder or other third-party litigation, as well as investigations by the SEC, the Nasdaq Global Select Market, or other regulatory authorities, which could require additional financial and management resources and could result in fines, trading suspensions, payment of damages or other remedies. Further, any delay in compliance with the auditor attestation provisions of Section 404 could subject us to a variety of administrative sanctions, including ineligibility for short-form resale registration, action by the SEC and the suspension or delisting of our common stock, which could reduce the trading price of our common stock and could harm our business.

We will continue to incur costs associated with satisfying our obligations as public company, and our management is required to devote substantial time to new compliance initiatives.

As a public company, and as a large accelerated filer we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act 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.

ITEM 1B. UNRESOLVED STAFF COMMENTS.

None.

ITEM 2. PROPERTIES.

Our corporate headquarters are located in New York, New York, where we currently lease 4,312 square feet pursuant to a lease agreement dated as of January 10, 2018, which expires five years from the date we first began to occupy the premises. In addition, we lease 4,783 square feet of combined office and laboratory space located in Nutley, New Jersey pursuant to a lease agreement dated as of February 11, 2019, as amended, which expires on February 10, 2022.

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

We believe that suitable additional or alternative space for both our U.S. and Danish locations would be available as required in the future on commercially reasonable terms.

ITEM 3. LEGAL PROCEEDINGS.

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently subject to any legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have an adverse effect on our business, operating results or financial condition.

ITEM 4. MINE SAFETY DISCLOSURES.

Not applicable.

PART II

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

Certain Information Regarding the Trading of Our Common Stock

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

On February 22, 2021, the last reported sale price for our common stock on the NASDAQ Global Select Market was $39.51 per share.

Holders of Our Common Stock

As of February 22, 2021 there were 13 holders of record of shares of our common stock. This number does not include stockholders for whom shares are held in "nominee" or "street" name.

Dividends

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

Securities authorized for issuance under equity compensation plans

Information about our equity compensation plans will be included in our definitive proxy statement to be filed with the SEC with respect to our 2021 Annual Meeting of Stockholders and is incorporated herein by reference.

Unregistered Sales of Equity Securities and Use of Proceeds

Recent Sales of Unregistered Equity Securities

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

In April 2020, we issued 42,900 shares of our common stock pursuant to a stock grant agreement entered into in connection with the entry into the SADA License Agreement. The issuance did not result in proceeds to the Company.

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

Use of Proceeds

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

initial public offering of $110.4 million, or aggregate net proceeds of approximately $99.8 million after deducting underwriting discounts and commissions and offering expenses. Merrill Lynch, Pierce Fenner & Smith Incorporated and Cowen and Company, LLC acted as joint book-running managers for the initial public offering. Canacord Genuity LLC acted as lead manager and BTIG LLC acted as co-manager for the initial public offering.

On November 1, 2019, we completed a secondary public offering of our common stock pursuant to which we issued and sold 5,134,750 shares of our common stock 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. We received aggregate gross proceeds from our secondary public offering of $143.8 million, or aggregate net proceeds of approximately $134.7 million after deducting underwriting discounts and commissions and offering expenses. Morgan Stanley, J.P. Morgan and BofA Securities acted as joint book-running managers for the secondary public offering. Wedbush PacGrow and H.C. Wainwright & Co. acted as co-managers for the secondary public offering.

On February 22, 2021, we completed a secondary public offering of our common stock pursuant to which we issued and sold 2,804,878 shares of our common stock at a price to the public of $41.00 per share which included the exercise in full of the underwriters' option to purchase additional shares. We received aggregate gross proceeds from our secondary public offering of $115.0 million, or aggregate net proceeds of approximately $107.7 million after deducting underwriting discounts and commissions and offering expenses J.P. Morgan, Morgan Stanley and BofA Securities acted as joint book-running managers for the secondary public offering. Kempen & Co. U.S.A. and H.C. Wainwright & Co. acted as co-managers for the secondary public offering.

None of the underwriting discounts and commissions or offering expenses were incurred or paid, directly or indirectly, to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any of our affiliates, and we have not used any of the net proceeds from the public offerings to make payments, directly or indirectly, to any such persons.

We have invested the net proceeds from the public offerings in cash and cash equivalents. There has been no material change in our planned use of proceeds as described in our final prospectus filed pursuant to Rule 424(b)(5) under the Securities Act with the SEC on October 31, 2019.

As of December 31, 2020, we had cash and cash equivalents of $114.6 million.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

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

ITEM 6. SELECTED FINANCIAL DATA.

The following tables set forth our selected consolidated financial data for the period indicated. We have derived the consolidated statements of operations data for the years ended December 31, 2020 and 2019 and the consolidated balance sheet data as of December 31, 2020 and December 31, 2019 from our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K. We have derived the selected financial data for the year ended December 31, 2018 from audited financial statements not included in this annual report. Our historical results are not necessarily indicative of the results that should be expected for any future period. You should read the following selected consolidated financial data together with the more detailed information contained in "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and the related notes included elsewhere in this Annual Report on Form 10-K.

	For the year ended December 31,		
	2020	2019	2018
	(in thousands, except per share data)		
Consolidated Statement of Operations Data:			
Revenue			
License revenue	$ 20,750	$ —	$ —
Operating expenses:			
Research and development	93,697	63,492	34,269
General and administrative	44,785	19,512	8,961
Royalties	2,203	—	—
Total operating expenses	140,685	83,004	43,230
Loss from operations	(119,935)	(83,004)	(43,230)
Interest and other income / (expenses)	598	1,976	(44)
Net loss	$ (119,337)	$ (81,028)	$ (43,274)
Net loss attributable to common stockholders	$ (119,337)	$ (81,028)	$ (43,274)
Net loss per share attributable to common stockholders—basic and diluted[(1)]	$ (2.97)	$ (2.30)	$ (1.50)
Weighted-average common shares outstanding used in computing net loss per share attributable to common stockholders—basic and diluted[(1)]	40,118,537	35,183,488	28,772,384

(1) See Note 4 to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K for an explanation of the method used to calculate the historical basic and diluted net loss per common share and the weighted average number of shares used in the computation of the per share amounts.

	December 31, 2020	December 31, 2019	December 31, 2018
		(in thousands)	
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 114,634	$ 207,136	$ 147,840
Working capital[(1)]	102,828	198,369	142,409
Total assets	132,047	216,366	151,924
Total liabilities	26,211	17,463	11,397
Accumulated deficit	(285,200)	(165,863)	(84,835)
Total stockholders' equity	105,836	198,903	140,527

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

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS.

You should read the following discussion and analysis of our financial condition and results of operations together with our accompanying financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. For convenience of presentation some of the numbers have been rounded in the text below.

Overview

We are a commercial-stage biopharmaceutical company focused on the development and commercialization of novel, antibody based therapeutic products for the treatment of cancer. We are leveraging our proprietary antibody platforms and deep expertise in the field of antibodies to develop a broad portfolio of innovative medicines.

On November 25, 2020, DANYELZA® (naxitamab-gqgk) was approved by the United States Food and Drug Administration, or the FDA, for the treatment, in combination with Granulocyte-Macrophage Colony-Stimulating Factor, or GM-CSF, of pediatric patients 1 year of age and older and adult patients with relapsed or refractory, or R/R, high-risk neuroblastoma, or NB, in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. DANYELZA is currently being investigated in three Phase 2 clinical studies for the treatment of patients with first-line NB, third-line NB, and in relapsed osteosarcoma. We are commercializing DANYELZA in the United States. In addition, we have an ongoing Phase 2 trial at Memorial Sloan Kettering Cancer Center, or MSK, with our GD2-GD3 Vaccine for the treatment of Stage 4 high-risk NB. We believe the GD2-GD3 Vaccine can potentially serve as an add-on treatment to DANYELZA.

We submitted a Biologics License Application, or BLA, to the FDA for radiolabeled 131I-omburtamab for central nervous system, or CNS, leptomeningeal metastases, or LM, from NB in August 2020, and received a Refusal to File letter from the FDA in October 2020. We are in the process of preparing a resubmission of the BLA and we plan to continue to discuss our resubmission plans with the FDA, including a meeting in March 2021 in order to amend the BLA. Assuming a positive outcome of these discussions, we expect to resubmit our BLA for omburtamab by the end of the second quarter of 2021 or the third quarter of 2021. We plan to commercialize omburtamab as soon as possible after obtaining FDA approval, if such approval occurs Additionally, we are conducting clinical studies with omburtamab in diffuse intrinsic pontine glioma, or DIPG, and desmoplastic small round cell tumor, or DSRCT. We also have an omburtamab follow-on product candidate, ^{177}Lu-omburtamab-DTPA, in Phase I for the treatment of medulloblastoma, and in Phase I in adults targeting B7-H3 positive CNS/LM tumors.

We are advancing a new generation of T cell engaging bispecific antibodies, or BsAbs, that may destroy tumor cells by recruitment of host T cells. Our Y-BiClone format contains two binding arms for the tumor target and two binding arms for T cells. This format was designed to have the minimal binding affinity necessary to recruit T cells. We have successfully opened an investigational new drug application, or IND, for our Phase 2 trial with nivatrotamab, our GD2 BsAb product candidate, in Small Cell Lung Cancer, or SCLC. In addition a Phase 1/2 trial with nivatrotamab, for the treatment of refractory GD2 positive adult and pediatric solid tumors is ongoing. Our nivatrotamab program thus addresses large patient populations. We are also advancing a CD33 BsAb for the treatment of hematological cancers expressing CD33, a transmembrane receptor expressed on cells of myeloid lineage, which we expect to enter clinical testing in 2021. We are advancing a pipeline of other novel BsAbs through late pre-clinical development. 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.

We are using our proprietary radioimmunotherapy platform to advance a series of antibody constructs based on the SADA technology, where bispecific antibody fragments bind to the tumor before a radioactive payload is injected in a two-step approach. We also refer to the SADA technology as Liquid Radiation™. We have designated GD2-SADA for potential use in GD2 positive solid tumors, B7-H3-SADA for potential use in prostate cancer, GPA33-SADA for potential use in colon cancer, and HER2-SADA for potential use in breast cancer as our first SADA constructs, and expect to file an IND for GD2-SADA in 2021. We believe the SADA technology could potentially improve the efficacy of radiolabeled therapeutics in tumors that have not historically demonstrated meaningful responses to radiolabeled agents.

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

Since our inception on April 30, 2015, we have devoted substantially all of our resources to organizing and staffing our company, business planning, identifying potential product candidates, conducting pre clinical studies of our product candidates and clinical trials of our lead product candidates, raising capital, and acquiring and developing our technology platform among other matters. We have not generated any substantial revenues from sales of DANYELZA which is currently our only approved product.

To date, we have financed our operations primarily through private placements of our securities, proceeds from our initial public offering and proceeds from our secondary public offering. On November 1, 2019, we completed our secondary public offering of 5,134,750 shares of our common stock, at a price of $28.00 per share, which includes the exercise in full of the underwriters' option to purchase 669,750 additional shares of our common stock. The aggregate gross proceeds to us, before deducting underwriting discounts and commissions and estimated offering expenses payable by us, were approximately $143.8 million. We have received aggregate gross proceeds of $375.8 million through December 31, 2020 from the sale and issuance of our common stock of $373.8 million and exercise of stock options of $2.0 million.

On February 22, 2021, we completed a secondary public offering of our common stock pursuant to which we issued and sold 2,804,878 shares of our common stock at a price to the public of $41.00 per share which included the exercise in full of the underwriters' option to purchase additional shares. We received aggregate gross proceeds from our secondary public offering of $115.0 million, or aggregate net proceeds of approximately $107.7 million after deducting underwriting discounts and commissions and offering expenses.

As of December 31, 2020, we had an accumulated deficit of $285.2 million. Our net losses were $119.3 million for the year ended December 31, 2020 and $81.0 million for the year ended December 31, 2019. 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 the regulatory approval process both in the U.S. and internationally;
- 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.

In conjunction with the approval by the FDA of DANYELZA in November 2020 we received a Priority Review Voucher, or PRV, which we subsequently sold to United Therapeutics Corporation in 2021 based on an agreed valuation of $105.0 million. We are obligated to pay 40% of the net proceeds to MSK. We intend to use the remaining proceeds to fund further research and development and other operational programs. The transaction closed in January 2021 upon the resolution of the substantive closing conditions, and has not impacted the income statement or balance sheet as per December 31, 2020. We do not expect to generate revenues from product sales unless and until we successfully launch DANYELZA.

Pursuant to the MSK License, we have obtained exclusive rights to MSK's rights in our current antibody product candidates. Under the MSK License, we have committed to funding scientific research at MSK as well as conducting certain clinical trial activities at MSK. 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 Master Data Services Agreement, or MDSA, and two separate Core Facility Service Agreements, or CFSAs. Also, under our Investigator-Sponsored Master Clinical Trial Agreement, or MCTA, with MSK, we will provide drug product and funding for certain clinical trials at MSK.

On April 15, 2020, we entered into a license agreement, or the SADA License Agreement, with MSK and Massachusetts Institute of Technology, or MIT, that grants us an exclusive, worldwide, sublicensable license to certain patent and intellectual property rights developed by MSK and MIT 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 using SADA-BiDE (2-step Self-Assembly and DisAssembly-Bispecific DOTA-Engaging antibody system) Pre-targeted Radioimmunotherapy Platform, or the SADA Technology, a concept we also refer to as Liquid Radiation™. The patents and patent applications covered by the SADA License Agreement are directed, in part, to the SADA Technology, as well as a number of SADA constructs developed by MSK. Upon entering into the SADA License Agreement in April 2020 and in exchange for the licenses, we paid MSK and MIT a cash upfront payment and issued an aggregate of 42,900 shares of our common stock to them.

As required under the SADA License Agreement, in October 2020, we entered into a Sponsored Research Agreement to fund at least $1,500,000 in scientific research at MSK over the next three years. Further, the SADA License Agreement requires us to pay to MSK and MIT 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, increasing to $60,000 once a patent has been issued, over the royalty term, commencing on the tenth anniversary of the license agreement. These amounts are non-refundable but are creditable against royalty payments otherwise due under the SADA License Agreement.

Under the SADA License, we are also obligated to pay MSK and MIT certain clinical, regulatory and sales-based milestone payments. 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 SADA License Agreement. Total clinical and regulatory milestones potentially due under the SADA License Agreement are $4,730,000 and $18,125,000, respectively. There are also sales-based milestones, totaling $23,750,000, that become due should the Company achieve certain amounts of sales of licensed products. In addition, for each of the SADA constructs generated by MSK and sold for the Company by a sublicensee, the Company may pay sales milestones in the total amount up to $60,000,000 based on the achievement of various cumulative net sales made by the sub-licensee. Finally, under the terms of the SADA License, MSK is entitled to receive 25% of any income generated from the sale of any PRV or the sale of other comparable incentives provided by any non-U.S. jurisdiction. The Company accrued $605,000 for milestones related to the SADA License in the year ended December 31, 2020.

These MSK agreements are important to our business. For a more detailed discussion of the terms and conditions of certain of these agreements, see Note 6 - License Agreements and Commitments.

If we obtain regulatory approval for our product candidates, we expect to incur significant milestone costs, as well as 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.

Recent Developments

Since it was first reported to have emerged in December 2019, a novel strain of coronavirus, which causes COVID-19, has spread around the world, including the New York metropolitan area and Copenhagen, Denmark, where our primary office and laboratory spaces are located. The coronavirus pandemic is evolving, and to date has led to the implementation of various responses, including government-imposed quarantines, travel restrictions and other public health safety measures. The extent to which the coronavirus impacts our operations or those of our third-party partners, including our preclinical studies, clinical trials, manufacturing operations and commercialization efforts, will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration of the outbreak, new information that will emerge concerning the severity of the coronavirus and the actions to contain the coronavirus or treat its impact, among others. We have taken temporary precautionary measures intended to help minimize the risk of the virus to our employees, including temporarily requiring all employees to work remotely, other than those performing or supporting business-critical functions, such as certain members of our laboratory staff, suspending all non-essential travel worldwide for our employees and employee attendance at industry events and in-person work-related meetings, which could negatively affect our business. For those employees that are performing or supporting business-critical functions, we have implemented stringent safety measures designed to comply with applicable US federal, state and local guidelines as well as Danish safety guidelines instituted in response to the COVID-19 pandemic. We cannot presently predict the scope and severity of the planned and potential shutdowns or disruptions of businesses and government agencies, such as the Securities and Exchange Commission, or SEC, the FDA or other domestic and international regulatory authorities.

Components of Our Results of Operations

License Revenue

The Company received FDA approval of DANYELZA in November 2020 and entered into exclusive licensing and distribution agreements for DANYELZA and omburtamab in certain international territories. License revenue consists of nonrefundable up-front payments received for the licensing rights as the company determined that the license was distinct from other promises within the arrangement. Refer to Note 3 – *Summary of Significant Accounting Policies* for additional detail.

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 Sponsored Research Agreements, or the SRA, the two CFSAs, the MCTA, and the MDSA, with MSK;
- expenses incurred under agreements with CROs, as well as investigative sites and consultants that conduct our non-clinical studies and pre-clinical and clinical trials;
- expenses incurred under agreements 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, 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 related to regulatory activities, including filing fees paid to regulatory agencies;
- outsourced professional scientific development services; and
- allocated expenses for utilities and other facility-related costs, including rent, insurance, supplies and maintenance expenses, and other operating costs.

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

- the number of clinical sites included in the trials;
- the availability and length of time required to enroll a sufficient number of suitable patients in our clinical trials;
- the actual probability of success for our product candidates, including the safety and efficacy, early clinical data, competition, manufacturing capability and commercial viability;
- significant and changing government regulation and regulatory guidance;
- the performance of our existing and any future collaborators;
- the number of doses patients receive;
- the duration of patient follow-up;
- the results of our clinical trials and pre-clinical studies;
- the establishment of commercial manufacturing capabilities;
- adequate ongoing availability of raw materials and drug substance for clinical development and any commercial sales;
- the timing of our BLA submissions and their acceptance;
- the receipt of marketing approvals, including a safety, tolerability and efficacy profile that is satisfactory to the FDA or any non-U.S. regulatory authority;
- any requirement by the FDA or any non-US regulatory authority to conduct post market surveillance or safety studies;
- 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 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 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 submissions for our pipeline candidates, including supplementary regulatory submissions for DANYELZA.

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, commercial, 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 costs associated with operating as a public company, including expenses related to services associated with maintaining compliance with exchange listing and the SEC requirements, director and officer insurance costs and investor and public relations costs. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses.

Royalty Expenses

Royalty expenses consist of royalty costs calculated as a percentage of license revenue per our licensing agreements.

Critical Accounting Policies and Significant Judgments and Estimates

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

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

Use of Estimates

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

The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition, including expenses, manufacturing, clinical trials, research and development costs and employee-related amounts, will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain it or treat COVID-19, as well as the economic impact on local, regional, national and international markets. We have made estimates of the impact of COVID-19 within our financial statements and there may be changes to those estimates in future periods. Actual results may differ from these estimates.

License Revenue

We have certain license agreements that are within the scope of Accounting Standards Codification 606, Revenue from Contracts with Customers (ASC 606), to determine the distinct performance obligations. We analogize to ASC 606 for the accounting for distinct performance obligations for which there is a customer relationship. Prior to recognizing revenue, we make estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur and when the uncertainty associated with the variable consideration is subsequently resolved. Total consideration may include nonrefundable upfront license fees, payments for research and development activities, reimbursement of certain third-party costs, payments based upon the achievement of specified milestones, and royalty payments based on product sales derived from the collaboration.

To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract, and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The company only applies the five-step model to arrangements that meet the definition of a contract with a customer under ASC 606, including when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the company assess the goods or services promised within each contract, determine those that are performance obligations, and assess whether each promised good or service is distinct. The company then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

If there are multiple distinct performance obligations, we allocate the transaction price to each distinct performance obligation based on our relative standalone selling price. In assessing whether a promised good or service is distinct in the evaluation of a license arrangement subject to ASC 606, the Company considers factors such as the research, manufacturing and commercialization capabilities of the licensing partner and the availability of the associated expertise in the general marketplace. The Company also considers the intended benefit of the contract in assessing whether a promised good or service is separately identifiable from other promises in the contract. If a promised good or service is not distinct, the Company combines that good or service with other promised goods or services until it identifies a bundle of goods or services that is distinct.

The standalone selling price is generally determined based on the prices charged to customers. We estimate the efforts needed to complete the performance obligations and recognize revenue by measuring the progress towards complete satisfaction of the performance obligations using input measures.

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 costs to obtain 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, CD33 License, MabVax Sublicense, and SADA License based upon the resolution of certain contingencies. Certain of these milestone payments are due and payable with the passage of time whether or not the milestones have actually been met. We record the milestone and royalty payment when the achievement of the milestone (including the passage of time) or payment of the milestone or royalty is probable and the amount of the payment is reasonably estimable.

Income Taxes

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

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

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

Stock-Based Compensation

We measure stock options granted to employees, directors, and consultants 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 for employees and directors. Forfeitures are accounted for as they occur. We issue stock options to employees and directors with only service-based vesting conditions and record the expense for these awards using the straight-line method over the requisite service period.

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 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.

Fair Value of Stock Options

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model.

The assumptions that the Company used to determine the fair value of the granted stock options were as follows:

- Risk-free interest rate: The risk-free interest rate assumption is based on the U.S. Treasury instruments whose terms were consistent with the expected option term of our stock options.

- Expected Dividend Yield: The expected dividend yield assumption is based on the fact that we have never paid cash dividends and have no present intention to pay cash dividends. Consequently, we used an expected dividend of zero.

- Expected Volatility: The expected stock price volatility is estimated by taking the average historic price volatility of industry peers and adjusting for differences in our life cycle and financing leverage. Our industry peers consist of several public companies in the biopharmaceutical industry.

- Expected Term: We determine the average expected life of stock options based on the simplified method in accordance with SEC Staff Accounting Bulletin Nos. 107 and 110. We expect to continue to use the simplified method until we have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term.

Results of Operations

Comparison of the Years Ended December 31, 2020 and 2019

The following table summarizes our results of operations for the years ended December 31, 2020 and 2019:

	Year Ended December 31,		
	2020	2019	Change
	(in thousands)		
Revenue:			
License revenue	$ 20,750	$ —	$ 20,750
Operating expenses:			
Research and development	93,697	63,492	30,205
General and administrative	44,785	19,512	25,273
Royalties	2,203	—	2,203
Total operating expenses	140,685	83,004	57,681
Loss from operations	(119,935)	(83,004)	(36,931)
Interest and other income	598	1,976	(1,378)
Net loss	$ (119,337)	$ (81,028)	$ (38,309)

License Revenue

License revenue was $20.8 million for the year ended December 31, 2020 and was attributable to revenue earned from outlicensing DANYELZA and omburtamab in Greater China and Israel. As part of the agreements, we received nonrefundable up-front fees of $20.8 million for the transfer of the license and know-how related to the product indications. We recognized the revenue as we determined the license to be distinct from other promises within the arrangement.

We did not have any revenues in the year ended December 31, 2019.

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, MIT, and non-employee researchers; and consumable costs, which are simultaneously deployed across multiple projects under development. These costs are included in the table below.

	Year Ended December 31,	
	2020	2019
	(in thousands)	
Outsourced manufacturing	$ 29,406	$ 30,478
Clinical trials	6,163	5,870
Outsourced research and supplies	17,034	15,466
Milestones and license acquisition costs	13,314	75
Personnel costs	13,339	6,512
Professional and consulting fees	2,789	1,686
Stock-based compensation	7,572	1,002
Other	4,080	2,403
	$ 93,697	$ 63,492

Research and development expenses increased by $30.2 million, from $63.5 million for the year ended December 31, 2019, to $93.7 million for the year ended December 31, 2020. This was primarily due to a $13.2 million

increase in Milestones and license acquisition cost related to the SADA agreement, including an upfront payment of $2.0 million, $3.3 million associated with equity issuances to MSK/MIT, $7.4 million for the issuance of shares to two non-employees, and $0.6 million for milestones which we considered probable for the period ending December 31, 2020. Outsourced services and supplies costs increased by $1.6 million for the year ended December 31, 2020, primarily obtained from MSK and CROs for DANYELZA and our lead product candidate omburtamab. Personnel-related costs including salary, benefits and non-cash stock-based compensation for personnel related to our research activities, increased by $13.4 million for the year ended December 31, 2020. The increase includes $4.8 million in stock-based compensation for a non-employee consultant who assisted with a development program. Professional and consulting fees increased by $1.1 million for the year ended December 31, 2020.

General and Administrative Expenses

General and administrative expenses increased by $25.3 million from $19.5 million for the year ended December 31, 2019 to $44.8 million for the year ended December 31, 2020. The increase in general and administrative expenses was primarily attributable to a $12.7 million increase in commercial expense incurred in conjunction with building up the sales and marketing infrastructure for the launch of DANYELZA and the potential launch of omburtamab; and a $8.9 million increase in employee-related costs, including salary, benefits and non-cash stock-based compensation for personnel related to our business activities. In addition, compared with 2019, insurance expenses increased by $2.1 million, and professional fees increased by $2.1 million in 2020.

Royalty Expense

Royalty expense was $2.2 million for the year ended December 31, 2019 and was attributable royalties owed related to the Sciclone and Takeda agreements discussed in License Revenue above. We did not have any royalty expenses in the year ended December 31, 2019.

Interest and Other Income

Interest and Other Income for the year ended December 31, 2020 decreased by $1.4 million from $2.0 million for the year ended December 31, 2019 to $0.6 million for the year ended December 31, 2020. Our interest income decreased by $1.4 million due to a decrease in interest rates foreign currency and exchange losses and in the current period.

Liquidity and Capital Resources

Overview

Since our inception we have incurred significant net operating losses and expect to continue to incur increasing net operating losses and significant expenses for the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We do not currently have any approved products and have never generated any revenue from product sales. We have financed our operations through December 31, 2020 primarily through gross proceeds from the sale of our common stock of $230.0 million in the years 2015 through 2018 and an additional $143.8 million from the sale of our common stock in 2019. As of December 31, 2020, we had cash and cash equivalents of $114.6 million. We will need additional capital to continue funding our operations, which we may obtain from additional equity or debt financings, collaborations, licensing arrangements, or other sources.

In conjunction with the approval by the FDA of DANYELZA in November 2020 we received a Priority Review Voucher, or PRV, which we subsequently sold to United Therapeutics Corporation based on an agreed valuation of $105.0 million. We were obligated to pay 40% of the net proceeds to MSK. We inted to use the remaining proceeds to fund further research and development and other operational programs. The transaction closed in January 2021 once all closing conditions had been met, and has not impacted the income statement or balance sheet as per December 31, 2020. We do not expect to generate revenues from product sales unless and until we successfully launch DANYELZA.

Cash Flows

The following table provides information regarding our cash flows for the years ended December 31, 2020 and December 31, 2019:

	Year Ended December 31,	
	2020	2019
Cash used in operating activities	$ (91,231)	$ (73,497)
Cash used in investing activities	(2,785)	(1,965)
Cash provided by financing activities	2,004	134,704
Effect of exchange rates on cash and cash equivalents	(490)	23
Net decrease in cash and cash equivalents	$ (92,502)	$ 59,265

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 $91.2 million during the year ended December 31, 2020, as compared to $73.5 million during the year ended December 31, 2019. The $17.7 million increase in net cash used in operations was primarily due to an increase in our net loss for the year ended December 31, 2020 of $38.3 million, compared to the year ended December 31, 2019. This increase was primarily due to an increase in our operating expenses in connection with the development of our lead product candidates, DANYELZA and omburtamab, and the expansion of our other business activities. Non-cash expenses included stock-based compensation to employees and non-employees, which increased by $20.6 million. Adjustments of working capital reflect an increase of $0.8 million for the year ended December 31, 2020 compared to the year ended December 31, 2019.

Net Cash Used in Investing Activities

Net cash used in investing activities was $2.8 million for the year ended December 31, 2020, as compared to $2.0 million for the year ended December 31, 2019. The $0.8 million increase in net cash used in investing activities was primarily caused by $2.6 million in loans to two researchers related to their individual tax payments due in conjunction with stock grant, refer to Note 7, “Stockholder’s Equity”. The increase was partially offset by decreased spending on laboratory equipment in 2020 as our New Jersey laboratory facilities were finalized in 2019.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $2.0 million during the year ended December 31, 2020, as compared to $134.7 million during the year ended December 31, 2019. The decrease in cash provided by financing activities was attributable to net proceeds of $134.7 million related to the issuance of common stock in the Company’s secondary public offering in the year ended December 31, 2019, which exceeded the $2.0 million net proceeds from exercise of stock options in the year ended December 31, 2020.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we complete clinical development of DANYELZA and omburtamab, and initiate and advance our BLA resubmission for omburtamab. 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 approval for any additional 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. 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.

In conjunction with the approval by the FDA of DANYELZA in November 2020 we received a PRV, which we subsequently sold to United Therapeutics Corporation based on an agreed valuation of $105.0 million. We were obligated to pay 40% of the net proceeds to MSK. We inted to use the remaining proceeds to fund further research and development and other operational programs. The transaction closed in January 2021, and has not impacted the income statement or balance sheet as per December 31, 2020.

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

- the scope, progress, timing, costs and results of clinical trials for developing our lead product candidates, naxitamab and omburtamab, and conducting pre-clinical studies and clinical trials for our other product candidates;
- research and pre-clinical development efforts for any future product candidates that we may develop;
- our ability to enter into and the terms and timing of any collaborations, licensing agreements, distribution 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;
- the amount and timing of future revenue, if any, received from commercial sales of our current and future product candidates upon any marketing approvals;
- proceeds received, if any, from monetization of any future PRVs;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the costs of operating as a public company.

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

additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

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

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Off-Balance Sheet Arrangements

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

Contractual Obligations and Commitments

Contractual obligations as of December 31, 2020 are related to payments of operating leases for a development, manufacturing and supply agreement with SpectronRx in South Bend, Indiana, and our office spaces at our corporate headquarters in New York, New York, a laboratory space located in Nutley, New Jersey, and Hørsholm, Denmark. Our obligations and commitments are disclosed in the contractual obligations table below:

	Payments Due By Period			
	Total	Less Than 1 Year	1 to 3 Years	4 to 5 Years
Operating Lease Commitments	$ 4,377,000	$ 2,180,000	$ 2,133,000	$ 64,000
Total	$ 4,377,000	$ 2,180,000	$ 2,133,000	$ 64,000

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

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

We have entered into three license agreements and certain other agreements with MSK. The license agreements are further described below as the MSK License, the MSK CD33 License, and the SADA License. Additionally, through the SAAA we have established a direct license with MSK relating to the GD2-GD3 Vaccine.

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, 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. The total expensed minimum royalty payments in 2016 under the MSK License were $1,200,000, upon determination that the payment of such minimum royalties was probable and the amount was estimable in 2016. We are also obligated to pay MSK certain clinical, regulatory and sales-based milestone payments under the MSK License and MSK CD33 License. Certain of the clinical and regulatory milestone payments become due at the earlier of completion of the related milestone activity or the date indicated in the MSK License. Total clinical, regulatory and sales based milestones potentially due under the MSK License are $2,450,000, $9,000,000 and $20,000,000, respectively. In addition, under the MSK CD33 License, we are obligated to make potential payments of $550,000, $500,000 and $7,500,000 for clinical, regulatory and sales based milestones, respectively. We record milestones in the period in which the contingent liability is probable and the amount is reasonably estimable.

On April 15, 2020, we entered the SADA License Agreement, which requires us to pay to MSK and MIT 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, increasing to $60,000 once a patent has been issued, over the royalty term, commencing on the tenth anniversary of the license agreement. These amounts are non refundable but are creditable against royalty payments otherwise due under the SADA License Agreement. Under the SADA License Agreement, we are also obligated to pay MSK and MIT certain clinical, regulatory and sales based milestone payments. 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 SADA License Agreement. Total clinical and regulatory milestones potentially due under the SADA License Agreement are $4,730,000 and $18,125,000, respectively. There are also sales based milestones, totaling $23,750,000, that become due should the Company achieve certain amounts of sales of licensed products. In addition, for each SADA construct generated by MSK and sold for the Company by a sublicensee, the Company may pay sales milestones in the total amount of $60,000,000 based on the achievement of various levels of cumulative net sales by the sublicensee. Further, under the SADA License Agreement, we have committed to funding scientific research at MSK for up to $1,500,000 over the next three years, which we will expense as incurred.

On December 2, 2019, we entered into the Settlement and Assumption and Assignment, or SAAA, of MSK License and Y-mAbs Sublicense Agreement, or the MabVax/Y-mAbs Sublicense, between us and MabVax dated June 27, 2018, with MabVax Therapeutics Holdings, Inc. and MabVax Therapeutics, Inc., or together, MabVax, and MSK, which became effective on December 13, 2019. Pursuant to the MabVax/Y-mAbs Sublicense, MabVax sublicensed to us certain patent rights and know-how for development and commercialization of products for the prevention or treatment of NB by means of administering a bi-valent ganglioside vaccine granted to MabVax, pursuant to an exclusive license agreement dated June 20, 2008 between MabVax and MSK, as amended, or the MabVax/MSK License Agreement.

On March 21, 2019, MabVax filed a voluntary petition for relief under Chapter 11 of the Bankruptcy Code. The essence of the transaction created by the SAAA was for us, in light of the Chapter 11 bankruptcy proceedings affecting MabVax, to preserve the MabVax/MSK License Agreement and the rights granted to us under the MabVax/Y-mAbs Sublicense and for us to create a direct relationship with MSK with respect to the rights covered under the MabVax/Y-mAbs Sublicense. Pursuant to the SAAA, MabVax agreed to assume the MabVax/Y-mAbs Sublicense and the MabVax/Y-mAbs License Agreement pursuant to Section 365 of the Bankruptcy Code and concurrently to assign both of these agreements to MSK. MabVax has also agreed to pay MSK a certain amount to cure all of its existing defaults under the MabVax/MSK License Agreement. We remain responsible for any potential downstream payment obligations by MabVax to MSK related to the GD2-GD3 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 GD2-GD3 Vaccine, then we are obligated to file with the FDA for

a PRV. The SAAA stipulates that, if we are granted a PRV from the FDA covering a licensed product under the MabVax/Y-mAbs Sublicense and the PRV is subsequently sold, we will pay directly to MabVax and to MSK, respectively, a percentage of the proceeds from the sale thereof in order that MabVax and MSK each receive the same amount therefrom as envisaged under the MabVax/MSK License Agreement. The MabVax/MSK License Agreement will expire with effect for us, on a country by country basis, on the later of the expiration of (i) 10 years from the first commercial sale of the licensed product in such country or (ii) the last to expire valid claim covering such licensed product rights at the time of and in the country of sale.

Research and development is inherently uncertain and, should such research and development fail, the MSK License, the MSK CD33 License, and SADA License are cancelable at our option. We have also considered the development risk and each party's termination rights under the three 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 CD33 License, the SADA License or the MabVax License. We have entered sublicenses with SciClone and Takeda under the MSK License in 2020. 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, the MSK CD33 License, or the SADA License with prior written notice to MSK.

Recent Accounting Pronouncements

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

Internal Controls and Procedures

We are required, pursuant to Section 404(a) of the Sarbanes Oxley Act, to furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment includes disclosure of any material weaknesses identified by management over our internal control over financial reporting. Our independent registered public accounting firm is required to report on the effectiveness of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley 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. Certain material weaknesses have been identified in our internal control over financial reporting. See the section herein entitled "Risk Factors"—We have 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. 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 completed an evaluation of our internal control over financial reporting, as required by Section 404, and concluded that our disclosure controls and procedures were not effective at the reasonable assurance level as of December 31, 2020, due to material weaknesses in our internal control over financial reporting.

We have engaged an independent registered public accounting firm to perform an audit of our internal control over financial reporting as of December 31, 2020, who has concluded that our internal controls over financial reporting are not effective as of December 31, 2020 due to material weaknesses identified.

We took various steps to address the material weaknesses identified which included the following during the year ended December 31, 2020:

- Hired additional professionals to enhance the depth and competence of our accounting and finance team and strengthened the overall oversight and review procedures with regards to segregation of duties, financial reporting, financial processes and procedures and overall internal control procedures;
- Implemented business process-level controls across all significant accounts and information technology general controls across all relevant domains. This included completing training such that the preparers and control operators had clear expectations as it relates to the control design, execution and monitoring of such controls, including enhancements to the documentation to evidence the execution of the controls;
- Enhanced controls relating to the posting of journal entries and the preparation of account reconciliations. Specifically, we have systematically enforced segregation of duties by restricting the ability of individuals to create and post certain journal entries and also implemented a secondary review of manual journal entries by an individual without the ability to post journal entries. Additionally, we have enhanced our account reconciliation process by incorporating standardized checklists and templates and also designed the process in a manner in which the reconciliations are reviewed by an individual without access to post journal entries;
- Designed the business-process and information technology controls such that appropriate segregation of duties are in place. We have also instituted periodic monitoring controls across relevant segregation of duties conflicts to assess whether access remains appropriate and necessary implemented compensating controls where access could not be restricted; and
- Strengthened our processes and controls surrounding the review of complex accounting transactions, including the preparation and review of such accounting memos.

Until the controls described above have been in place long enough for management to conclude that they are operating effectively, the material weakness described above will continue to exist.

We may not be able to complete our evaluation, testing or any required remediation in a timely fashion. During the evaluation and testing process I nsubsequent periods, 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.

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

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2020 and December 31, 2019, we had cash and cash equivalents of $114.6 million and $207.1 million, respectively. Due to the nature of our investments in money market funds, the carrying value of our cash equivalents approximate their fair value at December 31, 2020. 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. 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 highly rated securities including a Treasury money market fund. Due to the short-term nature of such balances, an immediate 100 basis point change in interest rates would not have any significant 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, or DKK, the currency used in the Kingdom of Denmark, where our wholly owned subsidiary, Y-mAbs Therapeutics A/S, is located. As of December 31, 2020 and December 31, 2019, we had cash and cash equivalents denominated in DKK of $(0.5) million and $1.1 million, respectively, and an immediate 2% change in DKK exchange rate would not have any material effect on the fair market value of cash balances with the subsidiary.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA.

Report of Independent Registered Public Accounting Firm

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

Opinions on the Financial Statements and Internal Control over Financial Reporting
We have audited the accompanying consolidated balance sheets of Y-mAbs Therapeutics, Inc. and its subsidiaries (the "Company") as of December 31, 2020 and 2019, and the related consolidated statements of net loss and comprehensive loss, of changes in stockholders' equity and of cash flows for the years then ended, including the related notes (collectively referred to as the "consolidated financial statements"). We also have audited the Company's internal control over financial reporting as of December 31, 2020, based on criteria established in Internal Control - Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of the Company as of December 31, 2020 and 2019, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America. Also in our opinion, the Company did not maintain, in all material respects, effective internal control over financial reporting as of December 31, 2020, based on criteria established in Internal Control - Integrated Framework (2013) issued by the COSO because material weaknesses in internal control over financial reporting existed as of that date related to the lack of 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 its financial statements; (b) design and maintain controls to 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.

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 the annual or interim financial statements will not be prevented or detected on a timely basis. The material weaknesses referred to above are described in Management's Report on Internal Control over Financial Reporting appearing under Item 9A. We considered these material weaknesses in determining the nature, timing, and extent of audit tests applied in our audit of the 2020 consolidated financial statements, and our opinion regarding the effectiveness of the Company's internal control over financial reporting does not affect our opinion on those consolidated financial statements.

Change in Accounting Principle

As discussed in Note 3 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

Basis for Opinions

The Company's management is responsible for these consolidated financial statements, for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting included in management's report referred to above. Our responsibility is to express opinions on the Company's consolidated financial statements and on the Company's internal control over financial reporting based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud, and whether effective internal control over financial reporting was maintained in all material respects.

Our audits of the consolidated financial statements included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

Definition and Limitations of Internal Control over Financial Reporting

A company's 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 for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Critical Audit Matters

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that (i) relates to accounts or disclosures that are material to the consolidated financial statements and (ii) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Recognition of Research and Development Costs related to Clinical Milestones under the SADA arrangement

As described in Note 3 to the consolidated financial statements, management estimated that the total research and development costs related to clinical milestones under the SADA agreement that became probable during the year ended December 31, 2020 was $0.6 million, and accordingly, accrued for these costs as non-current liabilities. The remaining clinical milestones, not considered probable, were $4.1 million as of December 31, 2020. Management records clinical milestone payments when the achievement of the milestones or payment of the milestones is deemed probable, and the amount of the payment is reasonably estimable. Specifically, management uses the collective clinical experience across the Company to determine likelihood of achievement, as well as the current stage of the compounds under development, and estimates the progress of its preclinical studies and clinical trials, completion of milestone events per underlying agreements, the time expected to complete certain development activities, each party's termination right under the license agreements, invoices received and contracted costs when evaluating whether the clinical milestones should be recognized in each reporting period.

The principal considerations for our determination that performing procedures relating to recognition of research and

development costs related to clinical milestones under the SADA agreement is a critical audit matter are the significant judgment by management when evaluating whether clinical milestones are probable in estimating the research and development costs to accrue or disclose in the reporting period; this in turn led to a high degree of auditor judgment, subjectivity and effort in performing procedures to evaluate the audit evidence obtained relating to whether the achievement of the licensing milestones was probable and the amount of the milestone payments were estimable. As described in the “Opinions on the Financial Statements and Internal Control over Financial Reporting” section, material weaknesses were identified which are related to this matter.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the consolidated financial statements. These procedures included testing the effectiveness of controls over management’s process relating to the recognition of research and development costs related to milestone payments, including controls over the determination of the estimate of milestones payments recognized at the initial accounting for the licensing agreements and controls monitoring the subsequent accounting for milestones payments. These procedures also included, among others, (i) testing management’s process for estimating research and development costs related to milestones payments for accrual or disclosure in the reporting period; (ii) evaluating the completeness and accuracy of underlying data provided by management; and (iii) evaluating the reasonableness of assumptions used in the estimate. Evaluating the reasonableness of assumptions used in the estimate involved assessing management’s ability to reasonably estimate milestone payments becoming probable by (i) obtaining supporting evidence for expected development activities and current stage of the compounds under development; (ii) evaluating the consistency of the assumptions with the underlying contractual terms of the arrangements, development plans and budgets; (iii) performing a comparison of the estimated length of time to complete preclinical and clinical studies for similar compounds or technologies, and (iv) reading communications from stakeholders on the development of the compounds.

/s/ PricewaterhouseCoopers LLP

Florham Park, New Jersey
March 1, 2021

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

Y-MABS THERAPEUTICS, INC.

Consolidated Balance Sheets

(in thousands, except share data)

	December 31, 2020	December 31, 2019
ASSETS		
CURRENT ASSETS		
Cash and cash equivalents	$ 114,634	$ 207,136
Other current assets	7,729	4,819
Total current assets	122,363	211,955
Property and equipment, net	1,825	2,052
Operating lease right-of-use assets	4,569	1,989
Other assets	3,290	370
TOTAL ASSETS	$ 132,047	$ 216,366
LIABILITIES AND STOCKHOLDERS' EQUITY		
LIABILITIES		
Accounts payable	$ 9,372	$ 8,520
Accrued liabilities	8,197	4,550
Operating lease liabilities, current portion	1,966	516
Total current liabilities	19,535	13,586
Accrued milestone and royalty payments	2,695	1,921
Operating lease liabilities, long-term portion	2,013	1,714
Other liabilities	1,968	242
TOTAL LIABILITIES	26,211	17,463
Commitments and contingencies (Note 6)		
STOCKHOLDERS' EQUITY		
Preferred stock, $0.0001 par value, 5,500,000 shares authorized at December 31, 2020 and December 31, 2019; none issued at December 31, 2020 and December 31, 2019	—	—
Common stock, $0.0001 par value, 100,000,000 shares authorized at December 31, 2020 and December 31, 2019; 40,688,447 and 39,728,416 shares issued at December 31, 2020 and December 31, 2019, respectively	4	4
Additional paid in capital	391,558	364,712
Accumulated other comprehensive income / (loss)	(526)	50
Accumulated deficit	(285,200)	(165,863)
TOTAL STOCKHOLDERS' EQUITY	105,836	198,903
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$ 132,047	$ 216,366

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

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Net Loss and Comprehensive Loss

(In thousands, except share and per share data)

	For the year ended December 31,	
	2020	2019
REVENUE		
License revenue	$ 20,750	$ —
OPERATING EXPENSES		
Research and development	93,697	63,492
General and administrative	44,785	19,512
Royalties	2,203	—
Total operating expenses	140,685	83,004
Loss from operations	(119,935)	(83,004)
OTHER INCOME, NET		
Interest and other income	598	1,976
NET LOSS	$ (119,337)	$ (81,028)
Other comprehensive income / (loss)		
Foreign currency translation	(576)	43
COMPREHENSIVE LOSS	$ (119,913)	$ (80,985)
Net loss per share attributable to common stockholders, basic and diluted	$ (2.97)	$ (2.30)
Weighted average common shares outstanding, basic and diluted	40,118,537	35,183,488

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

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Changes in Stockholders' Equity

(In thousands, except share data)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income / (Loss)	Accumulated Deficit	Stockholders' Equity
	Shares	Amount				
Balance December 31, 2018	34,193,666	$ 3	$ 225,352	$ 7	$ (84,835)	$ 140,527
Issuance of common stock to investors, net of issuance costs	5,134,750	1	134,703	—	—	134,704
Issuance of common stock to nonemployees	400,000	—	—	—	—	—
Stock-based compensation expense	—	—	4,657	—	—	4,657
Foreign currency translation	—	—	—	43	—	43
Net loss	—	—	—	—	(81,028)	(81,028)
Balance December 31, 2019	39,728,416	$ 4	$ 364,712	$ 50	$ (165,863)	$ 198,903
Exercise of stock options	299,706	—	2,004	—	—	2,004
Issuance of common stock to nonemployees	656,896	—	8,707	—	—	8,707
Stock-based compensation expense	3,429	—	16,135	—	—	16,135
Foreign currency translation	—	—	—	(576)	—	(576)
Net loss	—	—	—	—	(119,337)	(119,337)
Balance December 31, 2020	40,688,447	$ 4	$ 391,558	$ (526)	$ (285,200)	$ 105,836

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

Y-MABS THERAPEUTICS, INC.

Consolidated Statements of Cash Flows

(In thousands)

	Year ended December 31,	
	2020	2019
CASH FLOWS FROM OPERATING ACTIVITIES		
Net loss	$ (119,337)	$ (81,028)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	396	166
Stock-based compensation	16,135	4,657
Non-cash expense in connection with equity issuance to MSK/MIT	1,331	—
Non-cash expense in connection with equity issuance to inventors	7,376	—
Foreign currency transactions	(576)	43
Changes in assets and liabilities:		
Other current assets	(3,848)	(1,158)
Other assets	(311)	(182)
Accounts payable	852	2,617
Accrued liabilities and other	6,751	1,388
NET CASH USED IN OPERATING ACTIVITIES	(91,231)	(73,497)
CASH FLOWS FROM INVESTING ACTIVITIES		
Purchase of property and equipment	(175)	(1,965)
Loans to inventors	(2,610)	—
NET CASH USED IN INVESTING ACTIVITIES	(2,785)	(1,965)
CASH FLOWS FROM FINANCING ACTIVITIES		
Proceeds from issuance of common stock, net of issuance costs	—	134,704
Proceeds from exercised stock options	2,004	—
NET CASH PROVIDED BY FINANCING ACTIVITIES	2,004	134,704
Effect of exchange rates on cash and cash equivalents	(490)	23
NET DECREASE IN CASH AND CASH EQUIVALENTS	(92,502)	59,265
Cash and cash equivalents at the beginning of period	207,136	147,871
Cash and cash equivalents at the end of period	$ 114,634	$ 207,136
SUPPLEMENTAL DISCLOSURE OF NON-CASH ACTIVITIES		
Property and equipment purchases in accounts payable	—	49
Right-of-use assets obtained in exchange for lease obligations	2,679	901

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

Y-MABS THERAPEUTICS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

NOTE 1—ORGANIZATION AND DESCRIPTION OF BUSINESS

Y-mAbs Therapeutics, Inc. ("we," "us," "our," the "Company," or "Y-mAbs") is a commercial-stage biopharmaceutical company focused on the development and commercialization of novel, antibody based therapeutic products for the treatment of cancer. We are leveraging our proprietary antibody platforms and deep expertise in the field of antibodies to develop a broad portfolio of innovative medicines.

The Company is headquartered in New York and was incorporated on April 30, 2015 under the laws of the State of Delaware.

NOTE 2—BASIS OF PRESENTATION

The Company has 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 various stages of development. DANYELZA (naxitamab-gqgk) was approved by the U.S. FDA in November 2020, but there can be no assurance that the Company's other 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 from operations since inception and had an accumulated deficit of $285.2 million as of December 31, 2020 and $165.9 million as of December 31, 2019. Through December 31, 2020, the Company has funded its operations through proceeds from sales of shares of its common stock, including its initial public offering in September 2018 and its secondary public offering in November 2019.

As discussed in Note 12—Subsequent Events, after December 31, 2020, the Company announced the closing of its public offering of 2,804,878 shares of its common stock, at a public offering price of $41.00 per share, which includes the exercise in full of the underwriters' option to purchase 365,853 additional shares of common stock. The aggregate gross proceeds to Y-mAbs, before deducting underwriting discounts and commissions and estimated offering expenses payable by the Company, were approximately $115 million.

As of December 31, 2020, the Company had cash and cash equivalents of $114.6 million, and as of December 31, 2019 the Company had cash and cash equivalents of $207.1 million. As of the issuance date of the financial statements for the year ended December 31, 2020, the Company expects that its cash and cash equivalents at

December 31, 2020 will be sufficient to fund its operating expenses and capital expenditure requirements through at least the next twelve months, irrespective of whether any additional product approvals are obtained.

The Company may 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 on attractive terms or at all when needed from equity or debt financing. If FDA approval for omburtamab does not occur or is significantly delayed, and the Company is unable to obtain additional financing from these or other sources when needed, it will likely be necessary to take other actions to enhance its liquidity position which may include significantly reducing the current rate of spending through delaying, scaling back current operations, or suspending certain research and development programs and other operational programs.

The accompanying consolidated financial statements reflect the accounts of the Company and its wholly-owned subsidiary and have been prepared in accordance with accounting principles generally accepted in the United States of America (“GAAP”). All intercompany balances and transactions have been eliminated.

NOTE 3—SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Use of Estimates

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

The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition, including expenses, manufacturing, clinical trials, research and development costs and employee-related amounts, will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain it or treat COVID-19, as well as the economic impact on local, regional, national and international markets. We have made estimates of the impact of COVID-19 within our financial statements and there may be changes to those estimates in future periods. Actual results may differ from these estimates.

Cash and Cash Equivalents

The Company considers all highly liquid instruments with original maturities of three months or less from date of purchase to be cash equivalents. All cash and cash equivalents are held in highly rated securities including a Treasury money market fund which is unrestricted as to withdrawal or use. To date, the Company has not experienced any losses on its cash and cash equivalents. The carrying amount of cash and cash equivalents approximates its fair value due to its short-term and liquid nature. We maintain cash balances in excess of insured limits. We do not anticipate any losses with respect to such cash balances.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date (i.e. an exit price). The accounting guidance includes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The three levels of the fair value hierarchy are as follows:

• Level 1 — Unadjusted quoted prices for identical assets or liabilities in active markets;

• Level 2 — Inputs other than quoted prices in active markets for identical assets and liabilities that are observable either directly or indirectly for substantially the full term of the asset or liability; and

• Level 3 — Unobservable inputs for the asset or liability, which include management's own assumption about the assumptions market participants would use in pricing the asset or liability, including assumptions about risk.

Cash equivalents held in money market funds are valued using other significant observable inputs, which represent a Level 2 measurement within the fair value hierarchy. The Company has no other cash equivalents.

The following tables present the Company's fair value hierarchy for its cash equivalents, which are measured at fair value on a recurring basis (in thousands):

	Fair Value Measurements at December 31, 2020 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ —	$ 97,302	$ —	$ 97,302
	$ —	$ 97,302	$ —	$ 97,302

	Fair Value Measurements at December 31, 2019 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ —	$ 197,879	$ —	$ 197,879
	$ —	$ 197,879	$ —	$ 197,879

During the years ended December 31, 2020 and 2019, there were no transfers between Level 1, Level 2 and Level 3.

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.

Property and Equipment

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

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

Depreciation expense on property and equipment was $396,000 and $166,000 for the years ended December 31, 2020 and 2019, respectively.

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

Impairment of Long-Lived Assets

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

Income Taxes

The Company accounts for income taxes under the asset and liability approach for the financial accounting and reporting of income taxes. Deferred tax assets and liabilities are recognized for the estimated future tax consequences attributable to net operating loss carry forwards and temporary differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. These assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which the temporary differences are expected to reverse. We maintain a full valuation allowance on our deferred tax assets based on cumulative historical and expected losses. If we successfully commercialize our products and achieve profitability, we will consider the continued need for such valuation allowance.

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

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

In accordance with guidance issued by Financial Accounting Standards Board ("FASB"), companies should make and disclose a policy election as to whether they will recognize deferred taxes for basis differences expected to reverse as Global Intangible Low-Taxed Income ("GILTI") or whether they will account for GILTI as period costs if and when incurred. The Company has elected to recognize the resulting tax with respect to the GILTI provision as a period cost. No costs were incurred by the Company through December 31, 2020 as a result of GILTI.

Revenue Recognition

To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract, and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The company only applies the five-step model to arrangements that meet the definition of a contract with a customer under ASC 606, including when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the company assesses the goods or services promised within each contract, determines those that are performance obligations, and assess whether each promised good or service is distinct. The company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Under the practical expedient permitted under Topic 606, the Company expenses incremental costs of obtaining a contract as and when incurred if the expected amortization period of the assets is one year or less.

If there are multiple distinct performance obligations, we allocate the transaction price to each distinct performance obligation based on its relative standalone selling price. In assessing whether a promised good or service is distinct in the evaluation of a license arrangement subject to ASC 606, the Company considers factors such as the research, manufacturing and commercialization capabilities of the licensing partner and the availability of the associated expertise in the general marketplace. The Company also considers the intended benefit of the contract in assessing whether a promised good or service is separately identifiable from other promises in the contract. If a promised good or service is not distinct, the Company combines that good or service with other promised goods or services until it identifies a bundle of goods or services that is distinct. The standalone selling price is generally determined based on the prices charged to customers or using expected cost-plus margin.

The Company received FDA approval of DANYELZA in November 2020 and entered into exclusive licensing and distribution agreements for DANYELZA and omburtamab in certain international territories resulting in total license revenue of $20.8 million for the year ended December 31, 2020. Of this amount, we have $0.8 million as an outstanding receivable included within other current assets.

In December 2020, the Company entered into a development and commercialization arrangement with SciClone International Pharamceuticals Ltd. (SciClone) for certain indications of DANYELZA and omburtamab within China. As part of the agreement, we received a nonrefundable up-front fee of $20 million for the transfer of the license and know-how related to the product indications. The Company may receive regulatory-based milestone payments up to $40 million and sales-based milestone payments up to $60 million and is entitled to royalties based upon the net sales generated by SciClone related to the product indications in the territory. We considered the license to be distinct from other promises within the arrangement based on the rights and know-how transferred, late-stage development of the underlying indications and anticipated lack of significant involvement required from the joint steering committee associated with the indications. Accordingly, the full transaction price of $20 million was recognized upon transferring of the license and know-how to SciClone. The future potential regulatory milestone amounts were not included in the transaction price, as they were all determined to be fully constrained following the concepts of ASC 606. As part of our evaluation of the regulatory milestones constraint, we determined that the achievement of such milestones are contingent upon regulatory approvals which are not within our control and therefore not deemed probable. We expect that the sales-based milestone payments and royalty arrangements will be recognized when the related sales occur or the milestone is achieved. We will re-evaluate the transaction price each quarter and as uncertain events are resolved or other changes in circumstances occur, we assess whether this resolves the constraint and revenue will be recognized. We also considered that the manufacturing and supply terms, including within the arrangement, did not represent a material right to SciClone at inception as the terms reflected stand-alone selling price for similar goods or services. There was no license revenue generated in the period ended December 31, 2019.

In addition, the Company recorded $2.2 million in royalty expense which remained in accrued liabilities as of December 31, 2020 which are due to MSK based on the terms of the original MSK License agreement.

Research and Development Costs

Research and development costs are charged to operations when incurred and are included in operating expenses. Research and development costs consist principally of compensation cost for our employees and consultants that perform our research activities, the fees paid to maintain our licenses, the payments to third parties for manufacturing and clinical research organizations and additional product development, and consumables and other materials used in research and development. The Company records accruals for estimated ongoing research and development costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or clinical trials, including the phase or completion of events, invoices received and contracted costs. Actual results could differ from the Company's estimates. The Company is obligated to make certain milestone and royalty payments in accordance with the contractual terms of its license agreement with MSK based upon the resolution of certain contingencies. The Company records the milestone and royalty payments when the achievement of the milestone or payment of the milestone or royalty is probable and the amount of the payment is reasonably estimable. Research and development costs were $93.7 million and $63.5 million for the years ended December 31, 2020 and 2019, respectively.

Additionally, the Company is obligated to make certain royalty and clinical, regulatory and sales-based milestone payments in accordance with the contractual terms of the MSK License, CD33 License, MabVax Sublicense, and SADA License based upon the resolution of certain contingencies. Royalty payments and sales-based milestones are not due and deemed not probable as of December 31, 2020, as we have not recognized revenue for our only approved product, DANYELZA. We record the clinical and regulatory milestone payments when the achievement of the milestones or payment of the milestones is deemed probable, and the amount of the payment is reasonably estimable. As it relates to clinical and regulatory milestone payments under the SADA agreement, those may become due and payable with the passage of time whether or not the milestones have actually been met. When evaluating whether licensing milestones should be recognized under the SADA agreement, the Company uses its collective clinical experience across the company to determine the likelihood of achievement, as well as the current stage of the compounds under development, and estimates the progress of its preclinical studies and clinical trials, completion of milestone events per underlying agreements, the time expected to complete certain development activities, each party's termination right under the license agreements, invoices received and contracted costs when evaluating whether the clinical milestones should be recognized in each reporting period. We review our assessment each period and make revisions to such estimates as necessary. We recognized research and development costs in connection with SADA licensing milestones of $0.6 million for the year ended December 31, 2020 as we deemed them probable of occuring. The Company determined that the achievement of the regulatory milestones under the SADA agreement were not probable given the current stage of the product development. The remaining total clinical milestones of $4.1 million were determined to not be probable as of December 31, 2020, as detailed in Note 6 – *License Agreements And Commitments*.

Patent Costs

The Company expenses the costs of obtaining and maintaining patents as general and administrative expenses.

Advertising and Promotion Costs

Advertising and promotion costs are included in general and administrative expenses and were immaterial in the years ended December 31, 2020 and 2019. Advertising and product promotion costs are expensed as incurred.

Stock-Based Compensation

The Company measures stock options granted to employees, directors and consultants based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which for employees and directors is the vesting period of the respective award. Forfeitures are accounted for as they occur.

The Company issues stock options with only service-based or immediate vesting conditions and records the expense for these awards using the straight-line method over the requisite service period.

Following the Company's adoption of ASU 2018-07, Compensation—Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting ("ASU 2018-07"), on January 1, 2019, for stock-based option awards issued to non-employees, the Company no longer revalues non-employee awards at each reporting date and instead calculates the fair value of the awards as of the grant date using the Black-Scholes option-pricing model. The overall fair value measurement for non-employees awards is consistent with the awards issued to employees.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model. Prior to September 2018, the Company historically was a private company and lacks sufficient company-specific historical and implied volatility information for its shares. Therefore, it estimates its expected share price volatility based on the historical volatility of a group of publicly-traded peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded share price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards as the Company has limited historical data to support the expected term assumption. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield is based on the fact that the Company has never paid cash dividends on common shares and does not expect to pay any cash dividends in the foreseeable future.

Stock-based compensation costs were $16.1 million and $4.7 million for the years ended December 31, 2020 and 2019, respectively.

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.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity that result from transactions and economic events other than those with shareholders. The difference between net loss and comprehensive loss for the period presented in the accompanying financial statements was due to foreign currency translation.

Foreign Currency

The financial statements of our Danish subsidiary with a functional currency other than the U.S. dollar are translated into U.S. dollars using period-end exchange rates for assets and liabilities, historical exchange rates for stockholders' equity and weighted average exchange rates during the period for operating results. Translation gains and losses are included in accumulated other comprehensive income / (loss), net of tax, in stockholders' equity. Foreign currency transaction gains and losses are included in the results of operations in other income and expense, and totaled $(576,000) and $43,000 for the years ended December 31, 2020 and 2019, respectively.

Recently Issued Accounting Pronouncements - Adopted

In October 2020, the FASB issued Accounting Standards Update No. 2020-10 ("ASU 2020-10"), Codification improvements. The amendments in this Update represent changes to clarify the Codification, correct unintended application of guidance, or make minor improvements to the Codification. The adoption of this standard on December 31, 2020 did not have a material impact on our consolidated financial statements and related disclosures.

In August 2018, the FASB issued Accounting Standards Update No. 2018 -13 ("ASU2018-13"), Fair Value Measurement (Topic 820) Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement. ASU 2018-13 allows to remove the reasons for transfer between Level 1 and Level 2 assets, and adds the

changes in unrealized gains and losses for recurring level 3 fair value measurements. ASU 2018-13 is effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years with early adoption permitted. The adoption of this standard on January 1, 2020 did not have a material impact on our consolidated financial statements and related disclosures.

In August 2018, the FASB issued Accounting Standards Update No. 2018-15 ("ASU 2018-15"), Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract. ASU 2018-15 clarifies certain aspects of ASU 2015-05, Customer's Accounting for Fees Paid in a Cloud Computing Arrangement, which was issued in April 2015. Specifically, ASU 2018-15 aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal use software (and hosting arrangements that include an internal-use software license). ASU 2018-15 is effective for fiscal years beginning after December 15, 2019, and interim periods within those fiscal years with early adoption permitted. The adoption of this standard on January 1, 2020 did not have a material impact on our consolidated financial statements and related disclosures.

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 June 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update No. 2016-13 ("ASU 2016-13") Financial Instruments—Credit Losses (Topic 326): Measurement of credit losses on financial instruments. ASU 2016-13 introduces the current expected credit losses methodology (CECL) for estimating allowances for credit losses. The standard is effective in fiscal years and interim periods beginning after December 15, 2019. The adoption of this standard on January 1, 2020 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 most 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. Please refer to lease footnotes at Note 6 License Agreements And Commitments.

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:

	Year ended December 31,	
	2020	2019
Net loss (numerator)	$ (119,337)	$ (81,028)
Weighted-average shares (denominator)	40,119	35,183
Basic and diluted net loss per share	$ (2.97)	$ (2.30)

Potentially dilutive securities excluded from the computation of diluted earnings per share relate to stock options and unvested restricted share units (“RSUs”) outstanding totaled 5,704,246 shares as of December 31, 2020 and 4,005,873 shares as of December 31, 2019, and were excluded because including them would have an anti-dilutive impact.

NOTE 5—ACCRUED LIABILITIES

Accrued short-term liabilities are as follows:

	December 31, 2020	December 31, 2019
	(in thousands)	
Accrued licensing, milestone and royalty payments	$ 3,608	$ 354
Accrued clinical costs	678	1,584
Accrued compensation and board fees	2,603	1,475
Accrued manufacturing costs	983	760
Other	325	377
Total	$ 8,197	$ 4,550

NOTE 6—LICENSE AGREEMENTS AND COMMITMENTS

As of December 31, 2020, the Company has entered into two license agreements and certain other agreements with Memorial Sloan Kettering Cancer Center ("MSK"). The license agreements are the MSK License and the CD33 License Agreement. Through a Settlement and Assumption and Assignment of the MSK License and Y-mAbs Sublicense Agreement ("SAAA") with MabVax, Inc. ("MabVax") and MSK, the Company has established a direct license with MSK relating to the GD2-GD3 Vaccine, which was originally sublicensed by the Company in 2018 from MabVax.

In addition, the Company has entered into the SADA License Agreement with MSK and Massachusetts Institute of Technology ("MIT") in 2020. The license agreement with MSK and MIT grants the Company certain patent rights and intellectual property rights, and in consideration thereof, the Company agreed to make certain payments and issue shares of the Company's common stock to MSK and MIT.

Certain of the payments are contingent milestone and royalty payments, as disclosed in the table below. Amounts disclosed in Note 5 for accrued milestone and royalty payments are inclusive of obligations under the MSK License, CD33 License Agreement and SADA License Agreement, collectively. We have the following significant license agreements and related commitments which include all obligations that have been paid or are accrued as of and for the period ending December 31, 2020:

Agreements	Cash paid Twelve months ended December 2020	Cash paid Twelve months ended December 2019	Expense Twelve months ended December 2020	Expense Twelve months ended December 2019	Accrued liabilities Current as of December 2020	Accrued liabilities Non-current December 2020	Accrued liabilities Current as of December 2019	Accrued liabilities Non-current December 2019
MSK	$ 80,000	$ 725,000	$ -	$ 75,000	$ 305,000	$ 1,640,000	$ 254,000	$ 1,471,000
CD33	—	—	—	—	100,000	450,000	100,000	450,000
MabVax	—	600,000	—	—	—	—	—	—

The below table represents the maximum clinical, regulatory or sales-based miletones as reflected within the agreements, certain of which have been paid in prior periods or are accrued as presented in the table above:

Agreements	Maximum Clinical Milestones	Maximum Regulatory Milestones	Maximum Sales-based milestones
MSK	$ 2,450,000	$ 9,000,000	$ 20,000,000
CD33	550,000	500,000	7,500,000
MabVax	200,000	1,200,000	—

Minimum royalties and certain clinical milestones that become due based upon the passage of time under the CD33 License Agreement and the MabVax Agreement are not recorded as a liability as the Company does not consider such obligations to be probable as of December 31, 2020.

SADA License Agreement

On April 15, 2020, we entered into a license agreement (the "SADA License Agreement") with MSK and Massachusetts Institute of Technology ("MIT") that grants us an exclusive worldwide, sublicensable license to MSK's and MIT's rights to certain patent and intellectual property 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 using the SADA BiDE Pre-targeted Radioimmunotherapy Platform ("SADA technology"). We have assessed the licensing and other rights acquired and given the lack of outputs upon acquisition and that no employees were acquired, among other factors, we have concluded that the licensing rights represented an asset acquisition.

The patents and patent applications covered by this agreement are directed, in part, to the SADA technology, as well as a number of SADA constructs developed by MSK. Upon entering into the SADA License Agreement and in exchange for the licenses granted thereunder, we concluded that the technology acquired under the licensing arrangement had no alternative future use. This conclusion was based on consideration of the rights conveyed under the

agreement, extent of further development necessary and presence of uncertainty prior to obtaining regulatory approval for any product. Accordingly, we expensed and paid an upfront payment to MSK and MIT of $1,995,000. During the year ended December 31, 2020, we expensed $3,331,000 associated with stock grants available to MSK and MIT. This includes $1,331,000 related to 42,900 shares of common stock issued to the licensors on the effective date of the agreement based on the fair value of the stock on the grant date and $2,000,000 of future stock grants which will be paid on the anniversary date of the SADA License Agreement in 2021 and 2022. These awards survive the potential termination of the licensing arrangement, unless a breach by the licensors occurs, and can be settled in cash or stock at the determination of the Company. During the year ended December 31, 2020, we expensed $7,376,000 related to 213,996 shares issued to two non-employee researchers based on the fair value of the shares on the grant date. Please reference Note 7-Stockholders' Equity for additional details.

The SADA License Agreement requires us to pay to MSK and MIT 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, increasing to $60,000 once a patent has been issued, over the royalty term, commencing on the tenth anniversary of the license agreement. These amounts are non-refundable but are creditable against royalty payments otherwise due under the SADA License Agreement.

The Company is also obligated to pay MSK and MIT certain clinical, regulatory and sales-based milestone payments under the SADA 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 SADA License Agreement.

In addition, to the extent we enter into sublicense arrangements, we are obligated to pay to MSK and MIT a percentage of certain payments received from sublicensees of the rights licensed to us by MSK and MIT, which percentage will be based upon the achievement of certain clinical milestones. The Company has not entered into any sublicenses related to the SADA License Agreement. For each of the constructs previously generated by MSK using the SADA technology and sold for the Company by a sublicensee, the Company may pay sales milestones up to $60,000,000, in total, based on the achievement of various levels of cumulative net sales made by the sublicensee.

Failure by the Company to meet certain conditions under the arrangement could cause the related license to such licensed products to be canceled and could result in termination of the entire arrangement with MSK and MIT. In addition, the Company may terminate the SADA License Agreement with prior written notice.

Research and development is inherently uncertain and as described above, should such research and development fail, the SADA License Agreement is cancelable at the Company's option. The Company will also consider the development risk and each party's termination rights under the agreement when considering whether any clinical or regulatory based milestone payments, certain of which also contain time-based payment requirements, are probable. The Company records milestones in the period in which the contingent liability is probable and the amount is reasonably estimable. During the twelve months ended December 31, 2020, we expensed $605,000 of milestones under the SADA License Agreement. This includes all time-based milestones coming due within 36 months of the effective date of the agreement as this continues to represent the time period we expect will be required to gather necessary clinical data to determine which patent rights to further pursue, if any, under the SADA License Agreement. The Company does not consider any other milestones under the SADA License Agreement to be probable as of December 31, 2020.

We have the following SADA related balances and commitments which include all obligations that have been paid or accrued as of and for the priod ending December 31, 2020:

Agreements	**Cash paid** Twelve months ended December 2020	**Expense** Twelve months ended December 2020	**Expense** Twelve months ended December 2019	**Accrued liabilities** Current as of December 2020	**Accrued liabilities** Non-current as of December 2020	**Accrued liabilities** Current as of December 2019	**Accrued liabilities** Non-current as of December 2019
SADA	$ 2,707,000	$ 14,019,000	$ -	$ 1,000,000	$ 1,605,000	$ -	$ -

The below table represents the maximum clinical, regulatory or sales-based miletones as reflected within the agreements, certain of which have been paid in prior periods or are accrued as presented in the table above:

Agreements	Maximum Clinical Milestones	Maximum Regulatory Milestones	Maximum Sales-based milestones
SADA	$ 4,730,000	$ 18,125,000	$ 23,750,000

Minimum royalties and certain clinical milestones that become due based upon the passage of time under the SADA Agreement are not recorded as a liability as the Company does not consider such obligations to be probable as of December 31, 2020.

Other agreements

We have also entered into various other support agreements with MSK including a sponsored research agreement to provide research services related to the intellectual property licensed under the MSK License Agreement; a master data services agreement, for services provided by approximately five 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; a master clinical trial agreement pursuant to which we committed to fund certain clinical trials at MSK; two separate core facility service agreements pursuant to which we committed to obtaining certain laboratory services from MSK; and a CD33 sponsored research agreement 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; and in October 2020 we entered into a SADA sponsored research agreement pursuant to which we agreed to pay MSK to provide research services over a period of three years related to the intellectual property licensed under the SADA License Agreement. During 2020 and 2019, we incurred research and development expenses of $4.2 million and $6.8 million, respectively, under these agreements.

Lease Agreements

In July 2019, the Company entered a development, manufacturing and supply agreement with SpectronRx in South Bend, Indiana, to secure access to clinical and commercial scale radiolabeling capacity for omburtamab. Under the terms of the agreement, SpectronRx has agreed to establish a manufacturing unit designated for the Company within its existing facilities, at which both clinical and commercial supply of radiolabeled omburtamab can be produced. Since the Company possesses the right to substantially all the economic benefits and directs the use of the production area, the Company accounts for the payments related to the access to the manufacturing space under ASC 842 as an operating lease. The term of the lease is two years from the commencement date of August 31, 2020. Upon the lease commencement date, we recorded $3,617,000 as right-of-use asset and $2,680,000 as lease liability with the difference of $937,000 being due to prepayment of an initial fee of $500,000 to commence design and construction of the production area and access fees of $437,000. The company pays equal monthly installments of approximately $117,000 per month in additional access fees through September 2022 resulting in total payments of $2,330,000 remaining under the agreement. There are no renewal options in this agreement.

In February 2019, the Company entered into a lease agreement in connection with its 4,548 square feet laboratory in New Jersey. In December 2019, we expanded the space with an additional 235 square feet. 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.

January 2018, the Company entered into a lease agreement in connection with its corporate headquarters in New York. The term of the lease is five years from the date the Company begins to occupy the premises. Fixed rent payable under the lease is approximately $384,000 per annum and is payable in equal monthly installments of approximately $32,000, which are recognized on a straight-line basis.

Additionally, the Company entered a three-year lease agreement for the lease of certain office space in Denmark in February 2018, as amended in November 2018 and February 2019. The lease is payable in monthly installments of approximately $19,000, which are recognized on a straight-line basis.

As described above in Note 3, the Company adopted Topic 842 as of January 1, 2019.

Total operating lease costs were $1,324,000 and $676,000 for the year ended December 31, 2020 and 2019, respectively. During the year ended December 31, 2020, the expenses were recorded as $1,114,000 in research and development expense and $210,000 in general and administrative expense. During year ended December 31, 2019, the expenses were recorded as $493,000 in research and development expense and $183,000 in general and administrative expense. Cash paid for amounts included in the measurement of lease liabilities was $1,248,000 for year ended December 31, 2020 and $606,000 for year ended December 31, 2019, 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 December 31, 2020 and 2019 were as follows (in thousands):

	Operating Leases at December 31, 2020
2021	$ 2,180
2022	1,593
2023	540
2024	64
Total lease payments	4,377
Less: Imputed interest	(398)
Total operating lease liabilities at December 31, 2020	$ 3,979

Future minimum lease payments, including imputed interest, under non-cancelable operating leases at December 31, 2019 were as follows (in thousands):

	Operating Leases at December 31, 2019
2020	$ 749
2021	753
2022	646
2023	539
2024	77
Total lease payments	2,764
Less: Imputed interest	(534)
Total operating lease liabilities at December 31, 2019	$ 2,230

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 December 31, 2020, the weighted average remaining lease term is 2.18 years and the weighted average discount rate used to determine the operating lease liability was 7.6%. As of December 31, 2019, the weighted average remaining lease term is 3.83 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 December 31, 2020 and 2019, the Company has authorized a total of 105,500,000 shares, 100,000,000 of which are to be common stock, par value $0.0001 per stock, and 5,500,000 of which are to preferred stock, par value $0.0001 per share.

Common Stock

Each share of common stock is entitled to one vote. Common stockholders are entitled to receive dividends, as may be declared by the board of directors, if any, subject to preferential dividend rights of the preferred stock, none of which have been issued. The Company has issued 40,688,447 shares of its common stock as of December 31, 2020 and 39,728,416 shares of its common stock as of December 31, 2019.

Preferred Stock

Preferred stock may be issued from time to time in one or more series with such designations, preferences and relative participating, optional or other special rights and qualifications, limitations or restrictions as approved by the Company's Board of Directors. No preferred stock has been issued as of December 31, 2020 or December 31, 2019.

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. 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 544,000 shares were issued to the two researchers, whereby one of the two grants was fully issued. In 2019 a total of 400,000 shares were issued to one of the physicians, and the remaining 400,000 shares were issued in August 2020, subject to certain conditions. No future shares will be issued under this award. 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.

In April 2020, in connection with the SADA License Agreement, we entered into certain stock grant agreements pursuant to which we agreed to issue a total of 213,996 shares to two non-employee researchers who were involved in the development of the SADA technology licensed from MSK and MIT in consideration for their prior service. All 213,996 shares were issued in April 2020 into escrow with 40% of the shares immediately vesting at the time of issuance and the remaining 60% of the shares subject to vesting ratably over the next three years on the anniversary date of the agreement. The shares are subject to forfeiture to the extent the SADA License Agreement is terminated prior to the vesting of the shares. There is no cash settlement feature, and no future service is required for researchers to vest and receive the shares. While the shares vest over time, there is no performance condition for the shares. In April 2020, we recorded an expense within research and development totaling $7,376,000 related to the shares which represents the fair value of the shares on the grant date.

In July 2020, pursuant to the stock grant agreements, we also loaned the two researchers a total of $2,610,000 related to their individual tax payments due in conjunction with the stock grants. Each of the loans are evidenced by a three year Secured Promissory Note. The outstanding principal amounts of the loans, together with all accrued interest thereon at the rate of 1% per annum, is due and payable on the maturity date of the loans. The loans are secured by Pledge and Security Agreements, pursuant to which the researchers have pledged the shares as security for repayment of the loans with interest rates that are at market. The loans are recorded at amortized cost, which approximates fair value due to the short-term nature and minimal changes in market interest rates.

Issuance of common stock

In November 2019, we completed a secondary public offering and issued 5,134,750 shares of Common Stock at a purchase price of $28.00 per share for an aggregate consideration of $134,704,000, net of issuance costs of $9,100,000.

As dosclosed in Note 12, subsequent to December 31, 2020, on February 22, 2021, we completed a secondary public offering of our common stock pursuant to which we issued and sold 2,804,878 shares of our common stock at a price to the public of $41.00 per share which included the exercise in full of the underwriters' option to purchase additional shares. We received aggregate gross proceeds from our secondary public offering of $115.0 million, or aggregate net proceeds of approximately $107.7 million.

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 were 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 and Restricted Stock Units

During the years ended December 31, 2020 and 2019, stock-based compensation expenses for stock option grants were $15,949,000 and $4,581,000, respectively. During 2020 the expenses were recorded as $7,401,000 in research and development expense and $8,548,000 in general and administrative expense. During 2019 the expenses were recorded as $933,000 in research and development expense and $3,648,000 in general and administrative expense.

Other than $15,949,000 stock option compensation in 2020, another $186,000 restricted stock units, $169,000 in research and development expense and $17,000 in general and administrative expense were recorded in 2020. Other than $4,581,000 stock option compensation in 2019, another $76,000 restricted stock units, $69,000 in research and development expense and $7,000 in general and administrative expense were recorded in 2019. The total stock based compensation was $16,135,000 in 2020 and $4,657,000 in 2019, respectively.

Stock Option Valuation Activity

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

	Year Ended December 31, 2020	Year Ended December 31, 2019
Risk-free interest rate	0.64 %	1.78 %
Expected term (in years)	6.3	6.3
Expected volatility	65.0 %	60.6 %
Expected dividend yield	— %	— %

During the year ended December 31, 2020, the Company recognized $4.8 million in stock-based compensation related to an option grant of 150,000 options to a non-employee consultant for past service and contributions to a development program. No further service is required by the non-employee consultant to obtain the options and therefore the Company recorded the full value of the grant on the date of the grant.

The Company recognizes compensation expense for only the portion of awards that vest.

The following table summarizes common stock options issued and outstanding:

	Options	Weighted average exercise price	Aggregate intrinsic value (in thousands)	Weighted average remaining contractual life (years)
Outstanding and expected to vest at December 31, 2019	4,005,873	$ 10.67	$ 82,944	7.34
Granted	2,057,600	43.47		
Exercised	(299,706)	6.69		
Forfeited	(89,667)	25.00		
Outstanding and expected to vest at December 31, 2020	5,674,100	$ 22.55	$ 156,726	7.51
Exercisable at December 31, 2020	2,907,404	$ 8.42	$ 119,454	5.93

The weighted average grant-date fair value of stock options granted during the years ended December 31, 2020 and 2019 was $25.40 and $14.92 per share, respectively.

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company’s common stock for those stock options that had exercise prices lower than the fair value of the Company’s common stock. The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option pricing model. 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 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. There were no significant changes to the inputs included in the Black-Scholes option pricing model during the period ended December 31, 2020.

As of December 31, 2020 and 2019, we had $52,239,000 and $15,942,000, respectively, of unrecognized compensation related to employee stock options that are expected to vest over a period of 3.09 years in 2020 and 2.72 years in 2019.

Restricted Stock Unit Activity

The following table summarizes restricted stock units issued and outstanding:

	Restricted Stock Units	Weighted average grant price	Weighted average remaining vesting life (years)
Outstanding and expected to vest at December 31, 2019	10,296	$ 23.11	2.05
Granted	23,939	26.02	
Vested	(3,429)	23.11	
Forfeited	(660)	20.76	
Outstanding and expected to vest at December 31, 2020	30,146	$ 25.45	2.18

As of December 31, 2020, we had $586,000 of unrecognized compensation related to employee restricted stock units that are expected to vest over a period of 2.18 years. As of December 31, 2019, we had $163,000 of unrecognized compensation related to employee restricted stock units that are expected to vest over a period of 2.05 years.

NOTE 9—RELATED PARTY TRANSACTIONS

MSK is a shareholder of the Company. Under the MSK License Agreement, SADA License Agreement, the CD33 License Agreement, and various other supporting agreements with MSK, we have expensed costs in the total amount of $11,556,000 and $6,832,000 in the years ended December 31, 2020 and 2019, respectively, for milestones, minimum royalties, research and development costs, and patent activities. Please refer to Note 6 – License Agreements and Commitments for additional details on our agreements with MSK. As of December 31, 2020, we had a total of $833,000 recorded as accounts payable, $7,161,000 as accrued liabilities, thereby totaling $7,994,000 due to MSK. As of December 31, 2019, we had a total of $188,000 recorded as accounts payable, $3,983,000 as accrued liabilities, thereby totaling $4,171,000 due to MSK.

NOTE 10—INCOME TAXES

Domestic and foreign loss before income taxes are as follows:

	For The Year Ended December 31, 2020 (thousands)	For The Year Ended December 31, 2019 (thousands)
United States	$ (119,153)	$ (80,598)
Foreign	(184)	(430)
Total	$ (119,337)	$ (81,028)

The Company provided no current and deferred income tax benefits on net losses of $(119,337,000) and $(81,028,000) for years ended December 31, 2020 and 2019, respectively, and maintains a full valuation allowance against its net deferred tax assets.

The difference between income taxes expected at the U.S. federal statutory income tax rate of 21% for tax years ended December 31, 2020 and December 31, 2019, respectively, and income taxes provided are set forth below:

	December 31, 2020 (thousands)	December 31, 2019 (thousands)
Taxes on income at U.S. federal statutory rate	$ (25,061)	$ (17,016)
State and local taxes, net of federal tax effects	(3,059)	(11,022)
Effect of rate change	1,647	(22)
Foreign tax rate differential	(2)	(4)
Valuation allowance	30,986	33,984
Tax credits	(4,734)	(5,924)
Other	223	4
Total	—	—

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

	December 31, 2020 (thousands)	December 31, 2019 (thousands)
Deferred tax assets/(liabilities):		
Acquired intangibles	$ 4,424	$ 2,504
Accrued bonus	34	153
Unrealized foreign exchange loss	(448)	(261)
Accrued royalty	284	415
Stock based compensation	3,803	2,594
Net operating loss carryforwards	75,711	51,649
Tax credit carryforwards	13,146	8,412
ROU asset	(1,068)	(625)
Lease liability	931	711
Other	(357)	(63)
Total deferred tax assets/(liabilities)	96,460	65,489
Valuation allowance	(96,460)	(65,489)
Net deferred tax assets/(liabilities)	—	—

The Company recognizes income tax benefits for tax positions determined more likely than not to be sustained upon examination, based on the technical merits of the positions. As of December 31, 2020, and 2019, the Company has determined that there were no uncertain tax positions. The Company's tax returns for the years 2019, 2018, 2017 and 2016 are open for tax examination by U.S. federal and state, and the Danish tax authorities.

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

As of December 31, 2020, the Company had U.S. federal and state net operating loss ("NOL") carryforwards of approximately $248,942,000 and $168,792,000, respectively, which are available to reduce future taxable income. The

Company also had approximately $168,301,000 of unused NOL carryforwards for New York City purposes. The Company also had U.S. federal tax credits of $13,146,000 as of December 31, 2020, which may be used to offset future tax liabilities. The federal NOL carryforwards of approximately $29,909,000 will expire through 2037. The federal NOL of approximately $219,033,000 can be carried forward indefinitely but limited to offset 80% of taxable income. The Arizona, Massachusetts, Montana, New York State, and New York City NOL and tax credit carryforwards will begin to expire in 2035. The NOL and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986 (“IRC”). The Company has performed an analysis of its Section 382 ownership changes through December 31, 2018. Due to the large annual limitation, the Company believes that it is more likely than not that none of the net operating loss carryforwards will expire as a result of the limitation from the ownership change under Section 382. The Company also has Danish NOL carryforwards of $1,850,000, which have an indefinite carryforward period.

NOTE 11—OTHER BENEFITS

The Company has established a retirement program for employees of our Danish subsidiary pursuant to which all such employees can contribute an amount at their election from their base compensation and may receive contributions from our Danish subsidiary. Contributions from our Danish subsidiary were immaterial during the years ended December 31, 2020 and 2019. In addition, health insurance benefits for our Danish employees are fully paid for by such employees. Our Danish subsidiary does not incur any costs for these health insurance benefits.

On October 1, 2018, the Company adopted a defined contribution 401(k) savings plan (the “401(k) plan”) covering all U.S. employees of the Company. Participants may elect to defer a percentage of their pretax or after-tax compensation to the 401(k) plan, subject to defined limitations. The plan allows for a discretionary match by the Company. The Company made no matching contributions to the plan during the years ended December 31, 2020 and December 31, 2019.

NOTE 12 —SUBSEQUENT EVENTS

On December 28, 2020, the Company announced that it entered into a definitive agreement to sell its DANYELZA Priority Review Voucher to United Therapeutics Corporation for $105 million. The PRV was granted in conjunction with the approval by the U.S. Food and Drug Administration (“FDA”) of DANYELZA®, for the treatment of refractory/relapsed high-risk neuroblastoma. Under the terms of the Company’s license agreement with MSK, Y-mAbs will retain 60% of the net proceeds received from the sale, and the remaining 40% will be paid to MSK. The transaction closed on January 21, 2021 once the substantive closing conditions including within the agreement were resolved.

On February 22, 2021, the Company announced the closing of its public offering of 2,804,878 shares of its common stock, at a public offering price of $41.00 per share, which includes the exercise in full of the underwriters' option to purchase 365,853 additional shares of common stock. The aggregate gross proceeds to Y-mAbs, before deducting underwriting discounts and commissions and estimated offering expenses payable by the Company, were approximately $115 million.

NOTE 13—QUARTERLY CONSOLIDATED FINANCIAL DATA (unaudited)

(In thousands, except per share amounts)	2020			
	March 31	June 30	September 30	December 31
License revenue	$ —	$ —	$ —	$ 20,750
Loss from operations	(26,747)	(40,452)	(32,641)	(20,095)
Net loss	(26,179)	(40,393)	(32,832)	(19,933)
Net loss per share - basic and diluted	$ (0.66)	$ (1.01)	$ (0.82)	$ (0.48)

	2019			
	March 31	June 30	September 30	December 31
License revenue	$ —	$ —	$ —	$ —
Loss from operations	(16,253)	(18,634)	(24,359)	(23,758)
Net loss	(15,934)	(18,036)	(23,922)	(23,136)
Net loss per share - basic and diluted	$ (0.47)	$ (0.53)	$ (0.70)	$ (0.60)

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

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated, as of the end of the period covered by this Annual Report, the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act). Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were not effective at the reasonable assurance level as of December 31, 2020, due to material weaknesses in our internal control over financial reporting.

The design of any system of control is based upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated objectives under all future events, no matter how remote, or that the degree of compliance with the policies or procedures may not deteriorate. Because of their inherent limitations, disclosure controls and procedures may not prevent or detect all misstatements. Accordingly, even effective disclosure controls and procedures can only provide reasonable assurance of achieving their control objectives.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act). Under the supervision of and with the participation of our Chief Executive Officer and our Chief Financial Officer, our management conducted an assessment of the effectiveness of our internal control over financial reporting as of December 31, 2020 based on the framework and criteria established in *Internal Control – Integrated Framework*, issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework).

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.

Based on this assessment, management has concluded that as of December 31, 2020, our internal control over financial reporting was ineffective due to the following material weaknesses:

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) design and maintain controls to 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.

These material weaknesses did not result in material misstatements of the annual or interim consolidated financial statements; however, each of the material weaknesses described above could result in a misstatement of substantially all account balances or disclosures that would result in a material misstatement of our annual or interim consolidated financial statements that would not be prevented or detected.

Our independent registered public accounting firm has issued a report on the effectiveness of our internal control over financial reporting which is included under Item 8 *Financial Statements and Supplementary Data*.

Material Weakness Remediation Efforts

We took various steps to address the material weaknesses identified above which included the following during the year ended December 31, 2020:

- Hired additional professionals to enhance the depth and competence of our accounting and finance team and strengthened the overall oversight and review procedures with regards to segregation of duties, financial reporting, financial processes and procedures and overall internal control procedures;
- Implemented business process-level controls across all significant accounts and information technology general controls across all relevant domains. This included completing training such that the preparers and control operators had clear expectations as it relates to the control design, execution and monitoring of such controls, including enhancements to the documentation to evidence the execution of the controls;
- Enhanced controls relating to the posting of journal entries and the preparation of account reconciliations. Specifically, we have systematically enforced segregation of duties by restricting the ability of individuals to create and post certain journal entries and also implemented a secondary review of manual journal entries by an individual without the ability to post journal entries. Additionally, we have enhanced our account reconciliation process by incorporating standardized checklists and templates and also designed the process in a manner in which the reconciliations are reviewed by an individual without access to post journal entries;
- Designed the business-process and information technology controls such that appropriate segregation of duties are in place. We have also instituted periodic monitoring controls across relevant segregation of duties conflicts to assess whether access remains appropriate and necessary implemented compensating controls where access could not be restricted; and
- Strengthened our processes and controls surrounding the review of complex accounting transactions, including the preparation and review of such accounting memos.

Until the controls described above have been in place long enough for management to conclude that they are operating effectively, the material weaknesses described above will continue to exist.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting, (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the fourth quarter of the year ended December 31, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION.

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item 10 is incorporated by reference to the information set forth in the sections titled “Proposal 1 – Election of Directors,” “Executive Officers of the Company,” and “Information Regarding the Board and Corporate Governance” and “Section 16(a) Beneficial Ownership Reporting Compliance” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2021 Annual Meeting of Stockholders.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 is incorporated by reference to the information set forth in the section titled “Executive Compensation” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2021 Annual Meeting of Stockholders.

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

The information required by this Item 12 is incorporated by reference to the information set forth in the sections titled “Security Ownership of Certain Beneficial Owners and Management” and “Executive Compensation” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2021 Annual Meeting of Stockholders.

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

The information required by this Item 13 is incorporated by reference to the information set forth in the sections titled “Transactions with Related Persons” and “Information regarding the Board of Directors and Corporate Governance” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2021 Annual Meeting of Stockholders.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES.

The information required by this Item 14 is incorporated by reference to the information set forth in the section titled “Principal Accountant Fees and Services” in our Definitive Proxy Statement to be filed with the SEC with respect to our 2021 Annual Meeting of Stockholders.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES.

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

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

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

ITEM 16. FORM 10-K SUMMARY.

None.

EXHIBIT INDEX

Exhibit No.	
3.3	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.3 to the Form S-1 filed August 24, 2018).
3.4	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.4 to the Form S-1 filed August 24, 2018).
4.1	Specimen stock certificate evidencing the shares of common stock (incorporated by reference to Exhibit 4.1 to the Form S-1/A filed September 7, 2018).
4.2	Registration Rights Agreement, dated as of October 13, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.2 to the Form S-1 filed August 24, 2018).
4.3(a)	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.3(a) to the Form S-1 filed August 24, 2018).
4.3(b)	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.3(b) to the Form S-1 filed August 24, 2018).
4.3(c)	Registration Rights Agreement, dated as of November 17, 2017, among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.3(c) to the Form S-1 filed August 24, 2018).
4.4	Description of the registrant's securities registered pursuant to section 12 of the Securities Exchange Act of 1934.

10.1+	License Agreement, dated as of August 20, 2015, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.1 to the Form S-1 filed August 724, 2018).
10.2+	License Agreement, dated as of November 13, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.2 to the Form S-1 filed August 24, 2018).
10.3+	Sponsored Research Agreement, effective as of November 10, 2015, by and between the Registrant and Memorial Sloan Kettering Cancer Center Registrant (incorporated by reference to Exhibit 10.3 to the Form S-1 filed August 24, 2018).
10.4+	Sponsored Research Agreement, dated November 13, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.4 to the Form S-1 filed August 24, 2018).
10.5+	Investigator-Sponsored Master Clinical Trial Agreement, dated as of June 21, 2017, as amended on October 11, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.5 to the Form S-1 filed August 24, 2018).
10.6+	Master Data Services Agreement, dated as of September 23, 2016, as amended on October 11, 2017, by and between the Registrant and Memorial Sloan Kettering Cancer Center (incorporated by reference to Exhibit 10.6 to the Form S-1 filed August 24, 2018).
10.7†	Amended and Restated 2015 Equity Incentive Plan (incorporated by reference to Exhibit 10.7 to the Form S-1 filed August 24, 2018).
10.8†	Form of Notice of Grant and Stock Option Agreement under the Amended and Restated 2015 Equity Incentive Plan (incorporated by reference to Exhibit 10.8 to the Form S-1 filed August 24, 2018).
10.9†	2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.9 to the Form S-1 filed August 24, 2018).
10.10†	Form of Stock Option Grant Notice and Stock Option Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.10 to the Form S-1 filed August 24, 2018).
10.11†	Form of Officers and Directors Indemnification Agreement (incorporated by reference to Exhibit 10.11 to the Form S-1 filed August 24, 2018).
10.12†	Service Agreement, effective as of April 1, 2016 between the Registrant and Thomas Gad (incorporated by reference to Exhibit 10.12 to the Form S-1 filed August 24, 2018).
10.13†	Service Agreement, effective as of March 1, 2016 between the Registrant and Dr. Claus Juan Møller San Pedro, M.D., Ph.D. (incorporated by reference to Exhibit 10.13 to the Form S-1 filed August 24, 2018).
10.14†	Service Agreement, effective as of October 1, 2016 between Y-mAbs Therapeutics A/S and Bo Kruse (incorporated by reference to Exhibit 10.14 to the Form S-1 filed August 24, 2018).
10.15	Lease Agreement dated January 10, 2018, by and between the Registrant and RXR HB Owner LLC (incorporated by reference to Exhibit 10.15 to the Form S-1 filed August 24, 2018).

10.16†	Form of Restricted Stock Award Grant Notice and Restricted Stock Award Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.16 to the Form S-1 filed August 24, 2018).
10.17†	Form of Restricted Stock Unit Award Grant Notice and Restricted Stock Unit Award Agreement under the 2018 Equity Incentive Plan (incorporated by reference to Exhibit 10.17 to the Form S-1 filed August 24, 2018).
10.18†	Non-Employee Director Compensation Policy (incorporated by reference to Exhibit 10.18 to the Form S-1 filed August 24, 2018).
10.19†	Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.19 to the Form S-1 filed August 24, 2018).
10.20+	Amended and Restated Sponsored Research Agreement by and between the Registrant and Memorial Sloan Kettering Cancer Center effective September 13, 2019 (incorporated by reference to Exhibit 10.1 to Form 8-K filed September 19, 2019).
10.21+	Sublicense Agreement by and between the Registrant and MabVax Therapeutics Holdings, Inc. effective June 28, 2018 (incorporated by reference to Exhibit 10.1 to Form 8-K filed December 19, 2019).
10.22+	Settlement and Assumption and Assignment Agreement of MSK License Agreement and Y-mAbs Sublicense Agreement by and among the Registrant, MabVax Therapeutics Holdings, Inc. MabVax Therapeutics, Inc. and Sloan Kettering Institute for Cancer Research effective December 2, 2019 (incorporated by reference to Exhibit 10.2 to Form 8-K filed December 19, 2019).
10.23++	License Agreement effective as of April 15, 2020, by and among the Registrant, Memorial Sloan Kettering Cancer Center and Massachusetts Institute of Technology (incorporated by reference to Exhibit 10.1 to the Registrant's Form 8-K filed April 21, 2020).
10.24++	Master Sponsored Research Agreement by and between the Registrant and Memorial Sloan Kettering Cancer Center, effective October 7, 2020 (incorporated by reference to Exhibit 10.1 to the Registrant's Form 8-K filed October 8, 2020).
10.25	Form of Stock Option Grant Notice and Stock Option Agreement under the 2018 Equity Incentive Plan (as amended, employees, consultants and service providers other than directors) (incorporated by reference to Exhibit 10.8 to Registrant's Form 10-Q filed November 5, 2020).
10.26†	Form of Stock Option Grant Notice and Stock Option Agreement under the 2018 Equity Incentive Plan (as amended, directors) (incorporated by reference to Exhibit 10.9 to Registrant's Form 10-Q filed November 5, 2020).
10.27 ++	License Agreement dated December 17, 2020 by and between the Registrant and SciClone Pharmaceuticals International Ltd. (incorporated by reference to Exhibit 10.1 to Registrant's Form 8-K filed December 22, 2020).
10.28	Asset Purchase Agreement by and between the Registrant and United Therapeutics Corporation, dated December 24, 2020 (incorporated by reference to Exhibit 10.1 to Registrant's Form 8-K filed February 19, 2021).
21.1	Subsidiaries of the Registrant.
23	Consent of Pricewaterhouse Coopers LLP, Independent Registered Public Accounting Firm.

31.1	Certification of Chief Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act.
31.2	Certification of Chief Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act.
32.1*	Certification of Chief Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act.
32.2*	Certification of Chief Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act.
101.INS	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).

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

SIGNATURES

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

Y-MABS THERAPEUTICS, INC.

Dated: March 1, 2021

/s/ THOMAS GAD

Thomas Gad

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

POWER OF ATTORNEY

Each person whose signature appears below constitutes and appoints Thomas Gad, acting individually, as his or her true and lawful attorney-in-fact and agent, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or any of them, or their or his substitutes, may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Company and in the capacities indicated on the 25th of February 2021.

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

EXHIBIT 4.4

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

As of February 22, 2021, Y-mAbs Therapeutics, Inc. ("Y-mAbs," "we," "our" or "us"), had one class of securities registered under Section 12 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"): our common stock, par value $0.0001 per share.

The following summary describes our common stock and the material provisions of our amended and restated certificate of incorporation, our amended and restated bylaws, certain registration rights agreements (the "registration rights agreements") to which we and certain of our stockholders, including some of our directors and 5% stockholders and their affiliates and entities affiliated with our directors, are parties and of the Delaware General Corporation Law (the "DGCL"). Because the following is only a summary, it does not contain all of the information that may be important to you. For a complete description, you should refer to our certificate of incorporation, bylaws and registration rights agreements, filed as Exhibits 3.3, 3.4, 4.2, 4.3(a), 4.3(b) and 4.3(c), respectively, to our Annual Report on Form 10-K filed with the Securities Exchange Commission, of which this Exhibit 4.4 is a part. We encourage you to read those documents and the DGCL carefully.

Authorized capital stock

Our authorized capital stock consists of 100,000,000 shares of common stock, par value $0.0001 per share, and 5,500,000 shares of preferred stock, par value $0.0001 per share.

Common Stock

Outstanding shares

As of February 22, 2021, there were 43,526,254 shares of our common stock outstanding and no shares of preferred stock outstanding. As of February 22, 2021 we had 13 record holders of our common stock.

Voting Rights

Each holder of common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Our amended and restated certificate of incorporation and amended and restated bylaws do not provide for cumulative voting rights. Because of this, the holders of a plurality of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose. With respect to matters other than the election of directors, at any meeting of the stockholders at which a quorum is present or represented, the affirmative vote of a majority of the voting power of the shares present in person or represented by proxy at such meeting and entitled to vote on the subject matter shall be the act of the stockholders, except as otherwise required by law. The holders of a majority of the stock issued and outstanding and entitled to vote, present in person or represented by proxy, shall constitute a quorum for the transaction of business at all meetings of the stockholders.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and Preferences

Holders of common stock have no preemptive, conversion, subscription or other rights, and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate in the future.

Fully Paid and Nonassessable

All of our outstanding shares of common stock are fully paid and nonassessable.

Registration Rights

We are a party to the registration rights agreements (the "Registration Rights Agreements"), which provide the holders of registrable securities the right to require us to register such registrable securities under the Securities Act of 1933, as amended (the "Securities Act") under specified circumstances as described below. As of February 22, 2021, the shares subject to registration rights represent approximately 6 % of our outstanding common stock. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act.

Under the Registration Rights Agreements, holders of registrable shares can demand that we file a registration statement or request that their shares be included on a registration statement that we are otherwise filing, in either case, registering the resale of their shares of common stock. These registration rights are subject to conditions and limitations, including the right, in certain circumstances, of the underwriters of an offering to limit the number of shares included in such registration and our right, in certain circumstances, not to effect a requested S-1 or S-3 registration during the period that is 60 days before our estimated date of filing of, and ending on a date that is 90 days (or 180 days in the case of our initial public offering) after the effective date of, a company-initiated registration statement.

The registration rights of any holder terminate upon the earliest to occur of: (i) the date on which such holder holds no registrable shares, (ii) such time as Rule 144 or another similar exemption under the Securities Act is available for the sale of all of such holder's registrable shares without the requirement for us to be in compliance with the current publication information required under Rule 144(c)(1), and (iii) September 21, 2023, the fifth anniversary of our initial public offering.

Demand Registration Rights

Subject to specified limitations set forth in the Registration Rights Agreements, the holder or holders of not less than a majority of our registrable securities, as defined in the Registration Rights Agreements, acting together, may at any time demand in writing that we register the outstanding registrable securities under the Securities Act so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public of least $10 million. We are not obligated to file a registration statement pursuant to this demand provision on more than two occasions, subject to specified exceptions.

In addition, subject to specified limitations, a holder or holders of a majority of the registrable securities may demand in writing that we register on Form S-3 all or part of the registrable securities held by them so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public of least $10 million.

Incidental Registration Rights

If we propose to file a registration statement to register any of our common stock under the Securities Act, either for our own account or for the account of any of our stockholders that are not holders of registrable securities, and on a form that would also permit the registration of registrable securities, the holders of our registrable securities are entitled to notice of registration and, subject to specified exceptions, we will be required to use our best efforts to register the registrable securities then held by them that they request that we register.

Expenses of Registration

Pursuant to the Registration Rights Agreements, we are required to pay all registration expenses, including registration fees, printing expenses, fees and disbursements of our counsel and accountants and reasonable fees and disbursements not to exceed $50,000 of one counsel representing the selling stockholders, other than any underwriting discounts and commissions, related to any demand or incidental registration.

The Registration Rights Agreements contain customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

Preferred Stock

Our board of directors has the authority, without further action by the stockholders, to issue up to 5,500,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, redemption rights, liquidation preferences, sinking fund terms and the number of shares constituting any series or the designation of such series, any or all of which may be greater than the rights of common stock. The issuance of preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing change in our control or other corporate action. As of February 22, 2021, no shares of preferred stock were outstanding.

Anti-Takeover Provisions

Our amended and restated certificate of incorporation and amended and restated bylaws contain certain provisions that are intended to enhance the likelihood of continuity and stability in the composition of our board of directors but which may have the effect of delaying, deferring or preventing a future takeover or change in control of us unless such takeover or change in control is approved by our board of directors.

Staggered Board; Removal of Directors

Our amended and restated certificate of incorporation and amended and restated bylaws divide our board of directors into three classes with staggered three-year terms. In addition, our amended and restated certificate of incorporation and amended and restated bylaws provide that directors may be removed only for cause and only by the affirmative vote of the holders of $66^{2}/3\%$ of our shares of capital stock present in person or by proxy and entitled to vote. Under our amended and restated certificate of incorporation and bylaws, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office, although less than a quorum, or by a sole remaining director and shall not be filled by the stockholders. Furthermore, our amended and restated certificate of incorporation provides that the authorized number of directors may be changed only by the resolution of our board of directors. The classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Stockholder Action; Special Meeting of Stockholders; Advance Notice Requirements for Stockholder Proposals and Director Nominations

Our amended and restated certificate of incorporation and our amended and restated bylaws provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our amended and restated certificate of incorporation and amended and restated bylaws also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by the chairman of our board of directors, our chief executive officer or our board of directors. In addition, our amended and restated bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors, or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting

and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities. These provisions also could discourage a third party from making a tender offer for our common stock because even if the third party acquired a majority of our outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

Super-Majority Voting

The DGCL provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our amended and restated bylaws may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 66²/3% of the votes that all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 66²/3% of the votes that all our stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our amended and restated certificate of incorporation described above.

Section 203 of the Delaware General Corporation Law

We are subject to the provisions of Section 203 of the DGCL. In general, Section 203 prohibits a publicly-held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger, asset or stock sale or other transaction resulting in a financial benefit to the interested stockholder. An "interested stockholder" is a person who, together with affiliates and associates, owns, or did own within three years prior to the determination of interested stockholder status, 15% or more of the corporation's voting stock.

Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions: before the stockholder became interested, our board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder; upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances; or at or after the time the stockholder became interested, the business combination was approved by our board of directors of the corporation and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

Exclusive Forum Selection

Our 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 shall be the sole and exclusive forum for (1) any derivative action or proceeding brought on behalf of our company, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware, or (4) any action asserting a claim arising pursuant to any provision of our amended and restated certificate of incorporation or amended and restated bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine. This exclusive forum provision would not apply to suits brought to enforce any liability or duty created by the Securities Act or the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction. To the extent that any such claims may be based upon federal law claims, Section 27 of the Exchange Act creates exclusive federal jurisdiction over all suits brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. Although our amended and restated certificate of incorporation

contains the choice of forum provision described above, it is possible that a court could rule that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

Authorized but Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing requirements of the Nasdaq Global Select Market. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent and registrar's address is 6201 15th Avenue, Brooklyn, NY 11219.

Nasdaq Global Select Market

Our common stock is listed on the Nasdaq Global Select Market under the symbol "YMAB."

Exhibit 21.1

Subsidiaries of the Registrant

Y-mAbs Therapeutics A/S (Denmark)

Y-mAbs Therapeutics (Cayman Islands)

Note: Y-mAbs Therapeutics (Cayman Islands) is a dormant entity without any operations and holding no assets.

EXHIBIT 23

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statements on Form S-8 (No. 333-230455) and Form S-3 (No. 333-234034) of Y-mAbs Therapeutics, Inc. of our report dated March 1, 2021 relating to the financial statements and the effectiveness of internal control over financial reporting, which appears in this Form 10-K.

/s/ PricewaterhouseCoopers LLP

Florham Park, New Jersey
March 1, 2021

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

I, Claus Juan Møller San Pedro certify that:

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

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) 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. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: March 1, 2021

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

EXHIBIT 31.2

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

I, Bo Kruse, certify that:

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

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

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

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) 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. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

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

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

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

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

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

Date: March 1, 2021

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

EXHIBIT 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER

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

(i) the accompanying Annual Report on Form 10-K of the Company for the fiscal year ended December 31, 2020 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

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

Dated: March 1, 2021

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

EXHIBIT 32.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER

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

(i) the accompanying Annual Report on Form 10-K of the Company for the fiscal year ended December 31, 2020 (the "Report") fully complies with the requirements of Section 13(a) or Section 15(d), as applicable, of the Securities Exchange Act of 1934, as amended; and

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

Dated: March 1, 2021

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