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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**  
Pursuant to Section 13 or 15(d) of  
the Securities Exchange Act of 1934

Date of report (Date of earliest event reported): February 19, 2021 (February 17, 2021)

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**Y-MABS THERAPEUTICS, INC.**

(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

001-38650  
(Commission  
File Number)

47-4619612  
(I.R.S. Employer  
Identification No.)

230 Park Avenue  
Suite 3350  
New York, New York 10169  
(Address of principal executive offices) (Zip Code)

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

N/A  
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

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

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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.

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## ITEM 7.01 REGULATION FD DISCLOSURE.

### RISK FACTORS

*Your investment in our common stock involves a high degree of risk. Before deciding whether to invest in our common stock, you should carefully consider the risks discussed below and under the section captioned "Risk Factors" contained in our Annual Report on Form 10-K for the year ended December 31, 2019 and our Quarterly Report on Form 10-Q for the quarters ended March 31, 2020, June 30, 2020 and September 30, 2020, which were filed with the SEC and are incorporated by reference in this prospectus supplement and the accompanying prospectus in their entirety and which may be amended, supplemented or superseded by other reports we subsequently file with the SEC, together with other information in this prospectus supplement, the accompanying prospectus, the information and documents incorporated by reference herein and therein, and in any free writing prospectus that we have authorized for use in connection with this offering. If any of these risks actually occurs, our business, financial condition, results of operations or cash flow could be seriously harmed. This could cause the trading price of our common stock to decline, resulting in a loss of all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also impair our business operations. Please also read carefully the section entitled "Cautionary Statement Regarding Forward-Looking Statements."*

#### **Risks Related to this Offering**

***If you purchase shares of common stock in this offering, you will suffer immediate and substantial dilution in the book value of your investment.***

The price per share of our common stock in this offering is substantially higher than the net tangible book value per share of our common stock outstanding prior to this offering. Therefore, if you purchase shares of our common stock in this offering, you will incur immediate and substantial dilution in the net tangible book value per share of common stock from the public offering price per share of \$36.10. To the extent shares are issued under outstanding stock options at exercise prices lower than the price of our common stock in this offering, you will incur further dilution. See the section entitled "Dilution" for a more detailed illustration of the dilution you would incur if you participate in this offering.

***We have broad discretion in the use of our cash, cash equivalents and short-term investments, including the net proceeds we receive in this offering, and may not use them effectively.***

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

***You may experience future dilution as a result of future equity offerings.***

In order to raise additional capital, we may in the future offer additional shares of our common stock or other securities convertible into or exchangeable for our common stock at prices that may not be the same as the price per share in this offering. We may sell shares or other securities in any other offering at a price per share that is less than the price per share paid by investors in this offering, and investors purchasing shares or other securities in the future could have rights superior to existing stockholders. The price per share at which we sell additional shares of our common stock, or securities convertible or exchangeable for our common stock, in future transactions may be higher or lower than the price per share paid by investors in this offering.

*The price of our common stock has been and may be volatile in the future and fluctuate substantially.*

Our stock price has been and is likely to be volatile and may fluctuate substantially. 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. 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.

***Sales of a significant number of shares of our common stock in the public markets, or the perception that such sales could occur, could cause the market price of our common stock to drop significantly, even if our business is doing well.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have outstanding 42,911,460 shares of common stock based on (i) 40,472,435 shares outstanding as of September 30, 2020 and (ii) 2,439,025 shares of our common stock that we are selling in this offering, but not including any additional share issuable upon exercise of outstanding stock options or any exercise by the underwriters of their option to purchase additional shares from us. The shares that we are selling in this offering may be resold in the public market immediately without restriction, unless purchased by our affiliates.

We, our executive officers and directors and certain of our shareholders have agreed that, subject to certain exceptions, during the period ending 90 days after the date of this prospectus supplement, we and they will not offer, sell, contract to sell, pledge or otherwise dispose of, directly or indirectly, any of our common stock or securities convertible into or exchangeable or exercisable for any of our common stock, enter into a transaction that would have the same effect, or enter into any swap or other arrangement that transfers, in whole or in part, any of the economic consequences of ownership of our common stock, whether any of these transactions are to be settled by delivery of our common stock or other securities, in cash or otherwise, or publicly disclose the intention to make any offer, sale, pledge or disposition, or to enter into any transaction, swap, hedge or other arrangement, without, in each case, the prior written consent of the representatives of the underwriters, who may release any of the securities subject to these lock-up agreements at any time without notice. Exceptions to the lock-up restrictions are described in more detail in this prospectus supplement under the caption "Underwriters." J.P. Morgan Securities LLC, Morgan Stanley & Co. LLC and BofA Securities, Inc. may, in their discretion, permit us or our stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

These shares are eligible for sale in the public market under Rule 144 of the Securities Act, subject in certain cases to the volume limitations and other conditions of Rule 144 applicable to affiliates, after the earlier of the expiration of, or release from, the lock-up period. The holders of these shares may at any time decide to sell their shares in the public market.

Moreover, holders of an aggregate of approximately 2,744,000 shares of our common stock as of December 31, 2020, have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We have also registered all shares of common stock that we may issue under our equity compensation plans. As a result, these shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described above, to the extent applicable.

#### **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 we estimate net losses of approximately \$120.0 million for the year ended December 31, 2020. As of December 31, 2020 we estimate that our accumulated deficit will be approximately \$286.0 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. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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 <sup>131</sup>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.

Based upon preliminary estimates and information available to us as of the date of this prospectus supplement, we expect to report that we had approximately \$115.0 million of cash and cash equivalents as of December 31, 2020, see the section “Prospectus Supplement Summary—Financial Update.” We estimate that the net proceeds from this offering will be approximately \$93.6 million after deducting estimated offering expenses payable by us. We believe that such proceeds, together with our existing cash and cash equivalents, the proceeds we received in January 2021 from the sale of the PRV in connection with the approval of naxitamab, and assuming we receive FDA approval for omburtamab and the sale of the PRV related to such approval, will be sufficient to fund our operations through the fourth quarter of 2023. This financial information has been prepared by and is the responsibility of our management. Our independent registered public accounting firm, PricewaterhouseCoopers LLP, has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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, 2019 and 2018, it was determined that we lack a sufficient number of trained professionals with an appropriate level of accounting knowledge, training and experience to: (a) design and maintain formal accounting policies, procedures and controls over the fair presentation of our financial statements; (b) analyze, record and disclose complex accounting matters timely and accurately, including share based compensation arrangements and accounting for license arrangements; and (c) design and maintain controls over the preparation and review of account reconciliations, journal entries and financial statements, including maintaining appropriate segregation of duties.

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

We 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 will be 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 will be 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 will no longer be 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. As of the date of this offering, our independent registered public accounting firm has not finalized its work with respect to its audit of our financial statements or 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 and when that work is finalized, if the material weaknesses discussed above are not remediated or if additional material weaknesses related to our internal controls are identified, we may conclude that our internal controls are not effective and our independent registered public accounting firm will not be able to attest that our internal controls over financial reporting are effective pursuant to Section 404(b) of the Sarbanes-Oxley Act for the year ended December 31, 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 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<sup>®</sup> 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<sup>®</sup> in mainland China and in November 2020 EUSA and BeiGene announced that the BLA for QARZIBA<sup>®</sup> (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 or 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 prospectus supplement. 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 <sup>131</sup>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 <sup>131</sup>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, <sup>131</sup>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 nivatroamab 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 <sup>131</sup>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 United 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 40,668,447 shares of common stock outstanding as of December 31, 2020. Of these shares of our common stock, 6,900,000 shares sold in our initial public offering in 2018 and 5,134,750 shares sold in our public offering in 2019 are freely tradable, without restriction, in the public market. As of December 31, 2020 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. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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 21, 2018, our stock has traded at prices as low as \$14.16 per share and as high as \$55.22 per share through February 12, 2021. As a result of this volatility, you may not be able to sell your common stock at or above the price you 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 will be required to attest to the effectiveness of our internal control over financial reporting for the year ended December 31, 2020. We will no longer be 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. Preparing such attestation report and the cost of compliance with reporting requirements that we have not previously implemented will increase our expenses and require significant management time.

As of the date of this offering, our independent registered public accounting firm has not finalized its work with respect to its audit or our financial statements and when that work is finalized, there is no assurance that they may not issue a report on internal control over financial reporting that is adverse in the event our controls are determined to not be effectively designed, implemented or operating. 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 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.

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.

## OUR 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<sup>®</sup> (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, <sup>177</sup>Lu-omburtamab-DTPA, in Phase 1 for the treatment of medulloblastoma, and in Phase 1 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<sup>TM</sup>. 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 chemimmunotherapy 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. <sup>131</sup>I-omburtamab, which is omburtamab radiolabeled with Iodine-131, is currently being studied in several clinical trials including pivotal-stage development Study 101 and 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 <sup>131</sup>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. <sup>131</sup>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 <sup>131</sup>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.

<sup>124</sup>I-omburtamab, which is omburtamab radiolabeled with Iodine-124, is currently being studied for the treatment of Diffuse Intrinsic Pontine Glioma, or DIPG. <sup>131</sup>I-omburtamab is currently 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  $^{177}\text{Lu}$ -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  $^{177}\text{Lu}$ -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

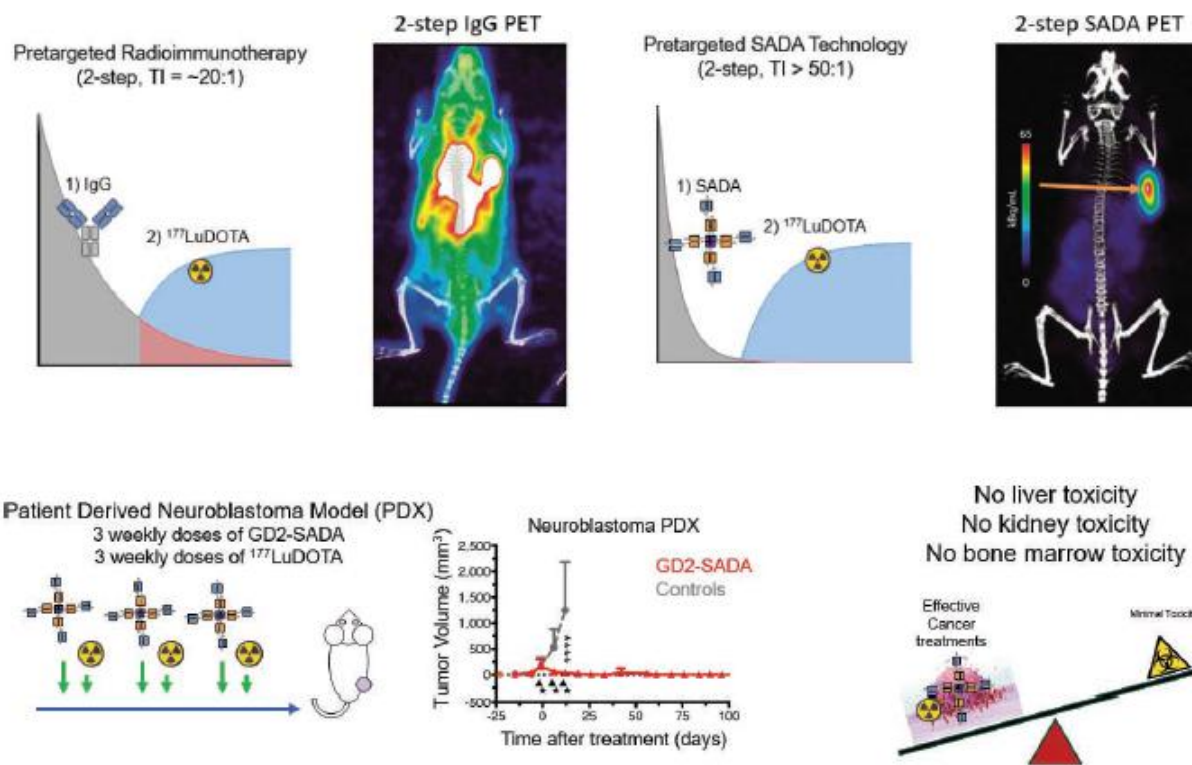
We currently have eight active INDs related to our product candidates. The table below sets forth the product candidate, date of the initial submission of the IND to the FDA, as well as the current sponsor, the subject matter and the current status of each such IND.

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 ( $^{131}\text{I}$ -omburtamab and $^{124}\text{I}$ -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
$^{177}\text{Lu}$ -omburtamab-DTPA	December 31, 2019	Y-mAbs	Medulloblastoma	Obtained IND in October 2020
$^{177}\text{Lu}$ -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<sup>TM</sup>. 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 approximately \$115.0 million. We have not yet completed our year-end financial close process for the year ended December 31, 2020. This estimate of our cash and cash equivalents as of December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. This preliminary estimate 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. There can be no assurance that our final cash position as of December 31, 2020 will not differ from these estimates, including as a result of review adjustments and any such changes could be material. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

## Our Pipeline

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

Product Candidate	Target	Study	Indication / Treatment	Preclinical	Phase 1	Phase 2	Phase 3 / Registration	Status/Next Anticipated Milestones
DANYELZA (naxitamab-gqgk)	GD2	201	Relapsed / Refractory High-Risk Neuroblastoma (Pediatric)	Ongoing pivotal Phase 2 trial <sup>1</sup>				Approved in November 2020
		12-230	Relapsed / Refractory High-Risk Neuroblastoma (Pediatric)	Phase 2 trial completed				
		16-1643	Front-Line High-Risk Neuroblastoma (Pediatric)	Ongoing Phase 2 trial				Multicenter Phase 2 to initiate in 2021 <sup>6</sup>
		15-096	Relapsed (Second-Line) Osteosarcoma <sup>2</sup>	Ongoing Phase 2 trial				
		17-251	Chemoimmunotherapy for Relapsed / Refractory High-Risk Neuroblastoma	Ongoing Phase 1 trial				Multicenter Phase 2 to initiate in 2021 <sup>6</sup>
Omburtamab	B7-H3	101	CNS / Leptomeningeal Metastases from Neuroblastoma (Pediatric) ( <sup>125</sup> I) <sup>3</sup>	Ongoing pivotal Phase 2 trial <sup>4</sup>				Q2/Q3 2021 BLA resubmission
		03-133	Intrathecal Immunotherapy for CNS/ Leptomeningeal Metastases ( <sup>125</sup> I) <sup>3</sup>	Ongoing Phase 1 trial				
		11-011	Diffuse Intrinsic Pontine Glioma (Pediatric) ( <sup>124</sup> T) <sup>3</sup>	Ongoing Phase 1 trial				Phase 2 to initiate in 2021
		19-182	Desmoplastic Small Round Cell Tumor (Pediatric) ( <sup>125</sup> I) <sup>3</sup>	Ongoing Phase 2 trial				
<sup>177</sup> Lu-omburtamab-DTPA <sup>5</sup>	B7-H3	301	Medulloblastoma	Ongoing Phase 1 trial				
		302	B7-H3 Positive CNS / Leptomeningeal Solid Tumors	Ongoing Phase 1 trial				
huB7-H3	B7-H3		Systemic Solid Tumors (Adult) (Third-Line)					
Nivatrotamab	GD2xCD3	402	Small Cell Lung Cancer	Ongoing Phase 2 trial				
		18-034	Refractory GD2-Positive Solid Tumors	Ongoing Phase 1 trial				
huCD33-BsAb	CD33xCD3		AML (Pediatric)					AML pediatric cancer IND in 2021
GD2-GD3 Vaccine	GD2-GD3	05-075	High-Risk Neuroblastoma patients in remission	Ongoing Phase 2 trial				Multicenter Phase 2 study to open in H2 2021
GD2-SADA	B7-H3		GD2 Positive Solid Tumors					IND 2021
GPA33-SADA	GPA33		Colon Cancer					IND 2022
HER2-SADA	HER2		Breast Cancer					IND 2022
B7-H3-SADA	B7-H3		Prostate Cancer					IND 2022

- (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) <sup>177</sup>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 BTB 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 <sup>131</sup>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 BTB granted in 2017. We expect to resubmit the BLA for <sup>131</sup>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 <sup>131</sup>I-omburtamab for pediatric CNS/LM from NB, we intend to discuss with the FDA the protocol for the continuation and expansion of the ongoing DIPG and DSRCT clinical trials. We believe that we may qualify for a supplemental BLA, or sBLA, in each of these indications assuming positive pivotal data.
- **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 <sup>177</sup>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 <sup>1</sup>/<sub>2</sub> 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 half-life as compared to e.g. BITE's hence allowing for weekly or even more sparse 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 BTM 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<sup>®</sup> 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 BTM 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

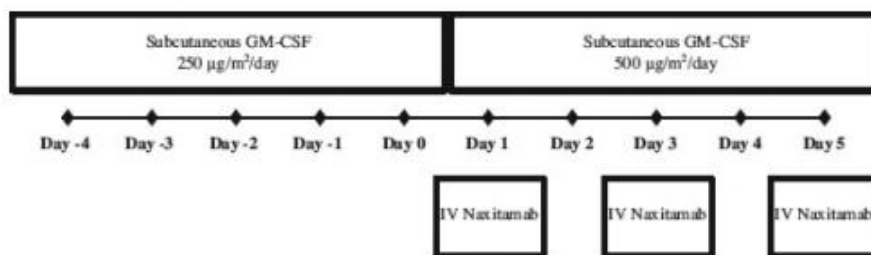
In addition to satisfying certain other criteria, patients must be over one year of age and must have been diagnosed with NB as defined by a) histopathology, or b) BM metastases or Meta-iodobenzylguanidine, or MIBG, avid lesion(s) plus high urine catecholamine levels.

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 123I-MIBG scans and who were deemed to have measurable disease and be eligible for response classification by the INRC classification incorporating 123I-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 123I-MIBG scan. Secondary refractory disease is defined as prior relapse and incomplete response to salvage therapy in BM as documented by histology and/or 123I-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 HAHA.

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<sup>2</sup>/d, divided into two doses, for 14 days. This treatment can be repeated after a minimum rest period of 14 days, for a total of six cycles starting after two cycles of naxitamab and GM-CSF unless HAHA develops and precludes timely administration of cycle 2 of naxitamab and GM-CSF. The interval between 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 an 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<sup>2</sup>/day IV will be administered from day one through five concurrently with temozolomide 150mg/m<sup>2</sup>/day orally. Naxitamab 2.25mg/kg IV will be administered on days two, four, eight and 10. Sargramostim 250mg/m<sup>2</sup>/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.

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 (<sup>131</sup>I-omburtamab) or Iodine-124 (<sup>124</sup>I-omburtamab). <sup>131</sup>I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB and was granted BTM in this indication in 2017. In 2016, <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>I-omburtamab and preliminary indications potentially suggest promising results.

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

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

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

<sup>131</sup>I-omburtamab is currently in pivotal stage development for the treatment of pediatric CNS/LM from NB, and was granted BTB in this indication in 2017. In 2016, <sup>131</sup>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 <sup>131</sup>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. <sup>131</sup>I-omburtamab can be administered as a push injection in an outpatient setting. We expect to resubmit the BLA for <sup>131</sup>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 <sup>131</sup>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.

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

There are currently no approved products for patients with R/R NB who have CNS/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 <sup>131</sup>I-omburtamab was first introduced in 2004, demonstrated a median OS of 5.5 months.

### *<sup>131</sup>I-omburtamab for Pediatric Central Nervous System/Leptomeningeal Metastases from Neuroblastoma—Clinical Development Program*

Currently, <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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, <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>I-omburtamab or <sup>124</sup>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)	
<b>TOTAL</b>	<b>177</b>		

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 <sup>131</sup>I-omburtamab treatment under Study 03-133. All patients had an intraventricular device implanted before enrollment in the study.

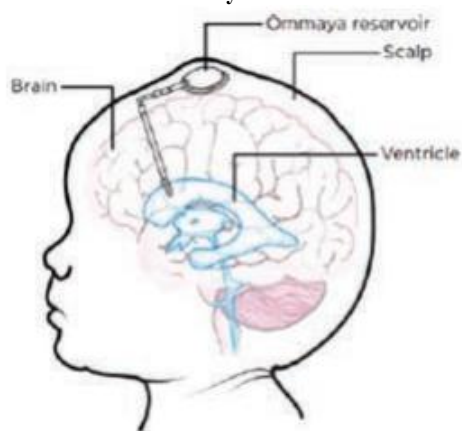
Approximately 80% of all CNS/LM from NB patients presenting at MSK since the initiation of the study were included in the study and the remaining patients were primarily excluded due to the fact that they had already received the maximum dose of previous radiotherapy to CNS, or had progressive systemic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

### Treatment Protocol

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

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

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



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



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



### Primary Objective

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

### Secondary Objective

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

### Safety Results

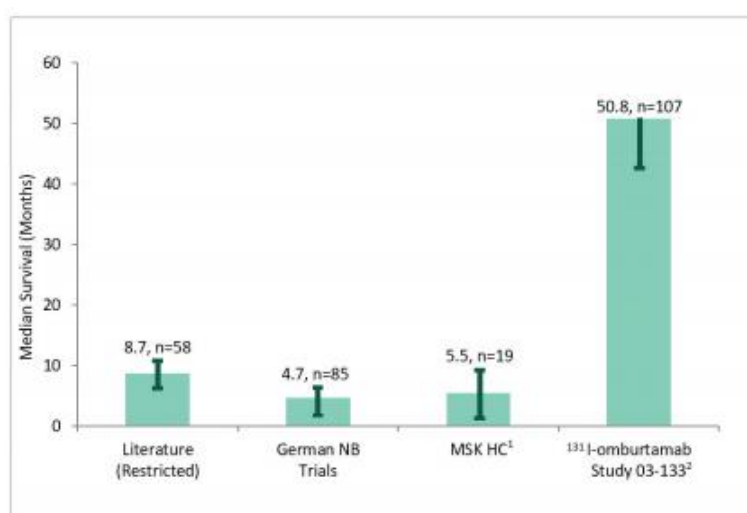
No MTD was reached in the dose escalation portion of the trial. Although not a DLT, myelosuppression was observed in patients who had received craniospinal radiation and <sup>131</sup>I-omburtamab at dose levels six and seven (60 and 70 mCi, respectively). As a result, a dose of 50 mCi was chosen for the expansion cohort. Among the 107 patients treated with <sup>131</sup>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 <sup>131</sup>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)

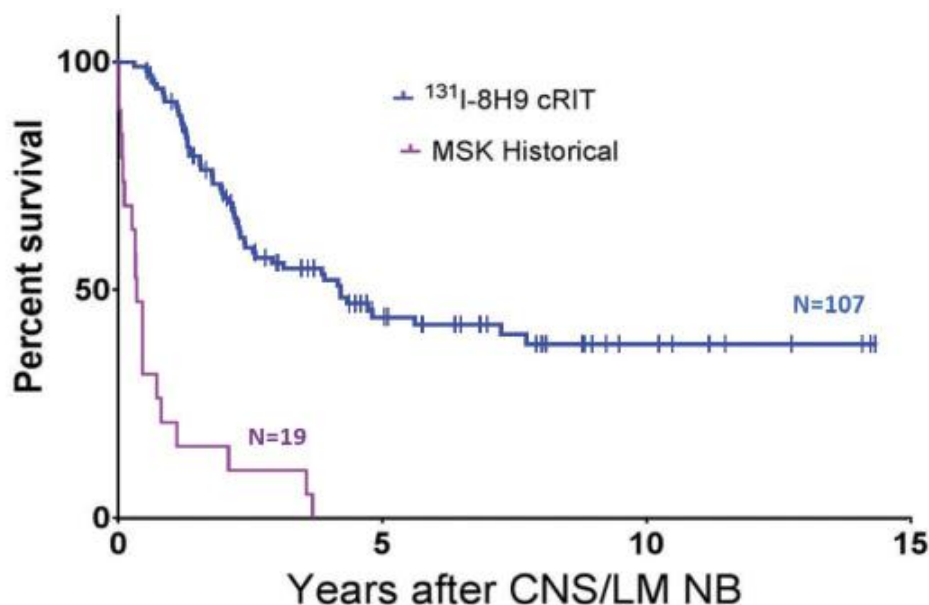


(1) MSK HC = NB patients with CNS / LM treated at MSK prior to 2003.

(2) <sup>131</sup>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 <sup>131</sup>I-omburtamab treatment. These results further demonstrate the lack of an established, effective therapy for these patients that we believe can potentially be addressed by <sup>131</sup>I-omburtamab.

The chart below shows the historical comparable data and median OS following the introduction of <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>I-omburtamab in pediatric patients with R/R NB who have CNS/LM from NB. Patients will receive up to two cycles of <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>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.

## *<sup>124</sup>I-omburtamab for the Treatment of Diffuse Intrinsic Pontine Glioma*

<sup>124</sup>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-<sup>124</sup> 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, <sup>124</sup>I-omburtamab received RPDD from the FDA for the treatment of DIPG. As of January 2021, we have treated 46 patients with DIPG with <sup>124</sup>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 <sup>124</sup>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.

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

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

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

<sup>124</sup>I-omburtamab 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 <sup>124</sup>I-omburtamab for Patients with Non-Progressive Diffuse Pontine Gliomas Previously Treated with External Beam Radiation Therapy*

MSK is conducting a Phase 1, dose escalation study of convection enhanced delivery, or CED, of <sup>124</sup>I-omburtamab in children with non-progressive DIPG previously treated with external beam radiation therapy. The study commenced in December 2011 and as of 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 <sup>124</sup>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 <sup>124</sup>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 <sup>124</sup>I-omburtamab administered via interstitial infusion in patients with DIPG.

#### *Secondary Objectives*

- To estimate tissue radiation doses and volumes of therapeutic distribution following <sup>124</sup>I-omburtamab interstitial infusion in the brainstem.
- To assess the toxicity profile associated with <sup>124</sup>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 <sup>124</sup>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 <sup>131</sup>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 <sup>131</sup>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.

## *<sup>131</sup>I-omburtamab for Treatment of Desmoplastic Small Round Cell Tumor*

<sup>131</sup>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.

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

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

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

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

<sup>131</sup>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 <sup>131</sup>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 <sup>131</sup>I-omburtamab for treatment of patients with DSRCT and other B7-H3 positive solid tumors metastatic to the peritoneum. The primary purpose of the study is to define the toxicity and the MTD, assess the pharmacokinetics, and assess response of DSRCT and other solid tumors. The study 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 <sup>131</sup>I-omburtamab reactive DSRCT or solid tumors that involve the peritoneum.

#### Treatment Protocol

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

#### *Primary Objective*

- To define the toxicity and the MTD of IP <sup>131</sup>I-omburtamab.

#### *Secondary Objectives*

- To assess pharmacokinetics for IP <sup>131</sup>I-omburtamab.
- To assess response of DSRCT and other solid tumors to IP <sup>131</sup>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 <sup>131</sup>I-omburtamab in this patient population.

*Study 19-182: A Phase 2 Study—Combination of <sup>131</sup>I-omburtamab Radioimmunotherapy and External Beam Radiotherapy for Desmoplastic Small Round Cell Tumors and Other Solid Tumors involving the Peritoneum*

This is an ongoing Phase 2 clinical study of IP RIT with <sup>131</sup>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 than DSRCT who are B7H3-positive on immunohistochemistry (Group C) will receive only IP <sup>131</sup>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 <sup>131</sup>I-omburtamab at a dose of 80mCi/m<sup>2</sup>. <sup>131</sup>I-omburtamab pharmacokinetics will be studied by a blood draw from indwelling venous lines. <sup>131</sup>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 <sup>131</sup>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.

### **<sup>177</sup>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 <sup>131</sup>I-omburtamab in 41 patients with tumors such as sarcoma, melanoma and medulloblastoma supports this objective. Our first such product candidate targeted towards larger patient populations is DTPA-conjugated omburtamab radiolabeled with Lutetium-177, which is currently in Phase 1 clinical development for the treatment of medulloblastoma and B7-H3 positive LM from solid tumors.

Animal toxicity studies of <sup>177</sup>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 <sup>177</sup>Lu-omburtamab-DTPA, can be distributed directly to hospitals, already conjugated and ready to use. It may then be administered to patients as a single-step push dose via an indwelling catheter for intracerebroventricular drug delivery, similar to the administration of <sup>131</sup>I-omburtamab in CNS/LM from NB. Therefore, we believe that a more easily available ready to use radiolabeled antibody such as <sup>177</sup>Lu-omburtamab-DTPA could be used more frequently, thereby significantly expanding our patient population beyond children.



On December 31, 2019, we submitted an Investigational New Drug application, or IND, for <sup>177</sup>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 <sup>177</sup>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.

#### *<sup>177</sup>Lu-omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases—Current Treatment Landscape and Associated Limitations*

Treatment of most patients with CNS/LM requires a combination of surgery, radiation, and/or chemotherapy. However, CNS/LM has 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.

### *<sup>177</sup>Lu-omburtamab-DTPA in Central Nervous System/Leptomeningeal Metastases—Mechanism of Action*

We are developing a Lutetium-177 conjugated omburtamab with DTPA as chelator. <sup>177</sup>Lu-omburtamab-DTPA will be administered to patients as a single-step push dose via an indwelling catheter for intracerebroventricular drug delivery, similar to the administration of <sup>131</sup>I-omburtamab in CNS/LM from NB. This form of administration will allow us to bypass the blood brain barrier and gain direct access to the CNS/LM. Lutetium-177 is a medium-energy beta-emitter with a maximal tissue penetration of 2 mm. Its half-life is approximately 6.7 days. Lutetium-177 also emits low-energy Gamma rays, which allows scintigraphy and subsequent dosimetry with the same therapeutic compound. Lutetium-177 is bound to omburtamab by DTPA. The resulting product <sup>177</sup>Lu-omburtamab-DTPA conjugate can be distributed 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: <sup>177</sup>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 <sup>177</sup>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 <sup>1</sup>/<sub>2</sub> 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 <sup>177</sup>Lu-omburtamab-DTPA

#### *Secondary Objectives*

- To assess absorbed radiation doses and organ dosimetry for <sup>177</sup>Lu-omburtamab-DTPA
- To evaluate the investigator assessed response, PFS and OS

### *Study 301: <sup>177</sup>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 <sup>177</sup>Lu-omburtamab-DTPA in patients with R/R medulloblastoma. We plan to enroll approximately 49 patients in this Phase <sup>1</sup>/<sub>2</sub> 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 <sup>177</sup>Lu-omburtamab-DTPA

#### *Secondary Objectives*

- To assess absorbed radiation doses and organ dosimetry for <sup>177</sup>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.

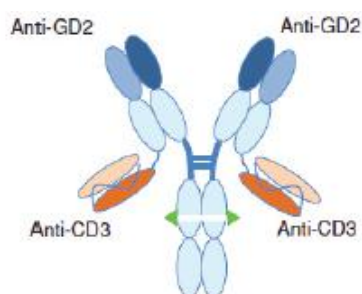
Non-IgG-like BsAbs lack a fragment crystallizable, or Fc, region, consisting instead of chemically linked variable regions and various types of multivalent single-chain variable fragments, or scFvs. One type of non-IgG-like BsAbs are BiTEs. BiTEs are relatively small and have more efficient penetration, 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)

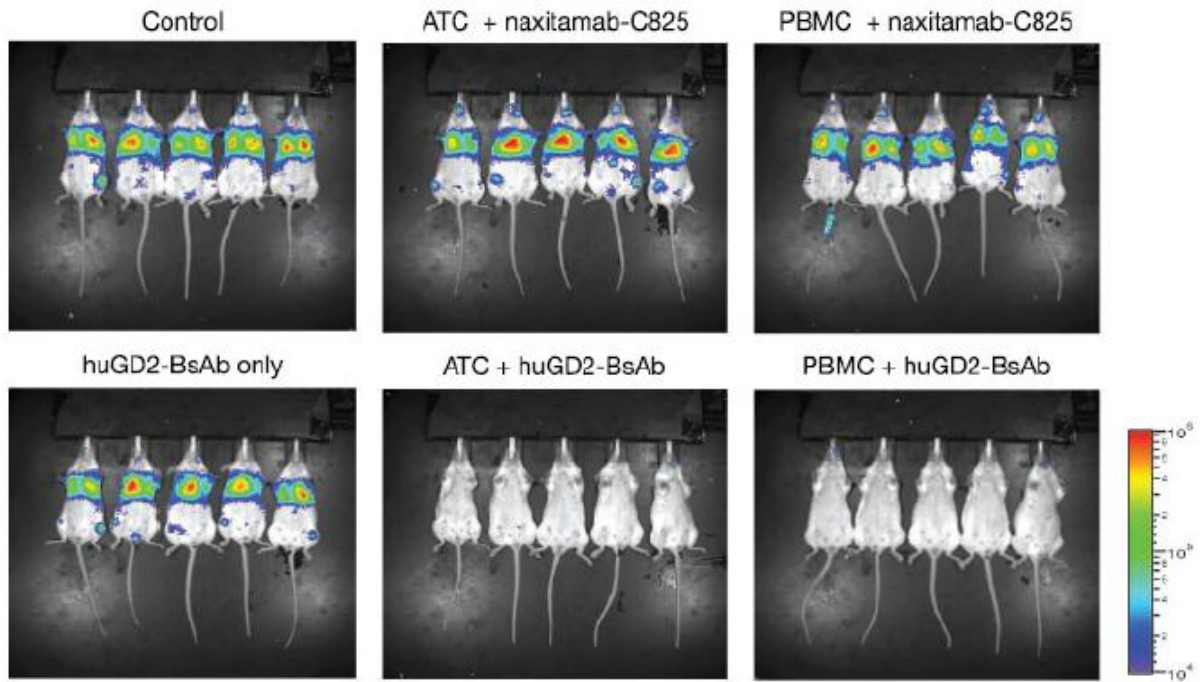


We believe that nivatrotamab may have several potential advantages over other BsAbs, including:

- 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 $\times$ 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  $\beta$ -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 IgG1 $\kappa$  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<sup>®</sup> 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<sup>®</sup> in mainland China and in November 2020 EUSA and BeiGene announced that the BLA for QARZIBA<sup>®</sup> (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 <sup>177</sup>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 enhancing 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 eighth 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. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section "Prospectus Supplement Summary—Financial Update" for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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 \$1,245,000, respectively, under the MDSA. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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 \$1,139,000 under the MCTA. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.



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 \$269,000, respectively, under the CFSAs. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section “Prospectus Supplement Summary—Financial Update” for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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. This financial data for the year ended December 31, 2020 is preliminary and is subject to change upon completion of our financial statement closing procedures. There can be no assurance that such financial data for the year ended December 31, 2020 will not differ from these estimates, including as a result of review adjustments, and any such changes could be material. The preliminary financial data included in this prospectus supplement has been prepared by and is the responsibility of our management. See the section "Prospectus Supplement Summary—Financial Update" for more information. PricewaterhouseCoopers LLP has not audited, reviewed, compiled, or applied agreed-upon procedures with respect to the preliminary financial data. Accordingly, PricewaterhouseCoopers LLP does not express an opinion or any other form of assurance with respect thereto. These results could change as a result of further review. Complete financial results for the year ended December 31, 2020 will be included in our Annual Report on Form 10-K.

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 <sup>131</sup>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<sup>TM</sup>. 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 BTM 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 may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received ODD. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

## **Rare Pediatric Disease Designation**

The Rare Pediatric Disease Priority Review Voucher Program, or the PRV Program, is intended to incentivize pharmaceutical companies to develop drugs for rare pediatric diseases. A company that obtains approval of an IND or a BLA for a designated rare pediatric disease may be eligible for a PRV from the FDA, which may be redeemed to obtain priority review for a subsequent new drug application or BLA by the owner of such PRV. A PRV is fully 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;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information on covered entities and their business associates that 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](http://www.ymabs.com). The information contained on, or accessible through, our website is not incorporated by reference into this prospectus supplement, and you should not consider any information contained in, or that can be accessed through, our website as part of this prospectus supplement or in deciding whether to purchase our common stock.

## ITEM 8.01 OTHER EVENTS.

On February 17, 2021, we entered into an underwriting agreement (the “Underwriting Agreement”) with J.P. Morgan Securities LLC, Morgan Stanley & Co. LLC and BofA Securities, Inc., as representatives of the several underwriters named therein (collectively, the “Underwriters”), relating to the issuance and sale in a public offering of 2,439,025 shares of our common stock, par value \$0.0001 per share. The price to the public in the offering is \$41.00 per share and the Underwriters have agreed to purchase the shares from us pursuant to the Underwriting Agreement at a price of \$38.54. In addition, we granted the Underwriters a 30-day option to purchase up to 365,853 additional shares of our common stock at the public offering price, less the underwriting discounts and commissions. The net proceeds to us from the offering are expected to be approximately \$93.6 million, or approximately \$107.7 million if the Underwriters exercise in full their option to purchase additional shares, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. The closing of the offering is expected to occur on February 22, 2021, subject to the satisfaction of customary closing conditions. We currently anticipate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, to (i) fund clinical development in additional pediatric and adult indications for DANYELZA<sup>®</sup>; (ii) fund clinical development in additional pediatric indications and expansion into adult B7-H3 positive cancers for omburtamab; (iii) fund our SADA technology programs, currently in pre-clinical development, into clinical testing in a range of indications; (iv) fund our lutetium-labeled B7-H3 antibody, 177Lu-omburtamab-DTPA, into late-stage clinical testing in a range of pediatric and adult indications, including medulloblastoma and B7-H3 positive adult cancers; (v) fund clinical development of ivatrotamab, our bi-specific GD2 antibody, currently in Phase 2 for SCLC and take our portfolio of BsAb product candidates, including huCD33-BsAb for pediatric acute myeloid leukemia, or AML, into clinical testing; (vi) fund ongoing and expanding clinical development through regulatory submission of our GD2-GD3 Vaccine program; (vii) fund commercial infrastructure build-out for launch and other commercial activities for both DANYELZA and omburtamab, if approved; (viii) fund translational science, pre-clinical research and clinical development activities related to our other product candidates, programs and in-licensing of new technologies; and (ix) the remainder for working capital and other general corporate purposes.

The Underwriting Agreement contains customary representations, warranties and agreements by us, customary conditions to closing, indemnification obligations of us and the Underwriters, including for liabilities under the Securities Act of 1933, as amended, other obligations of the parties and termination provisions. The representations, warranties and covenants contained in the Underwriting Agreement were made only for purposes of such agreement and as of specific dates, were solely for the benefit of the parties to such agreement, and may be subject to limitations agreed upon by such parties.

The offering is being made pursuant to our registration statement on Form S-3 (Registration Statement No. 333-234034), which was declared effective by the Securities and Exchange Commission (“SEC”) on October 15, 2019, and a prospectus supplement thereunder. The foregoing description of the Underwriting Agreement is not complete and is qualified in its entirety by reference to the full text of the Underwriting Agreement, a copy of which is filed as Exhibit 1.1 to this Current Report on Form 8-K and is incorporated by reference herein. A copy of the opinion of Duane Morris LLP relating to the legality of the issuance and sale of the shares in the offering is attached as Exhibit 5.1 hereto.

On February 16, 2021, we issued a press release announcing the offering, and on February 17, 2021, we issued a press release announcing that we had priced the offering. Copies of these press releases are attached as Exhibits 99.1 and 99.2 hereto, respectively.

**ITEM 9.01 FINANCIAL STATEMENTS AND EXHIBITS.**

(d)

<b>Exhibit No.</b>	<b>Description</b>
<a href="#">1.1</a>	<a href="#">Underwriting Agreement, dated February 17, 2021, by and among Y-mAbs Therapeutics, Inc., and J.P. Morgan Securities LLC, Morgan Stanley &amp; Co. LLC and BofA Securities, Inc., as representatives of the several underwriters named therein.</a>
<a href="#">5.1</a>	<a href="#">Opinion of Duane Morris LLP</a>
<a href="#">10.1</a>	<a href="#">Asset Purchase Agreement by and between Y-mAbs Therapeutics, Inc. and United Therapeutics Corporation dated December 24, 2020</a>
<a href="#">23.1</a>	<a href="#">Consent of Duane Morris LLP (included in Exhibit 5.1)</a>
<a href="#">99.1</a>	<a href="#">Press Release, dated February 16, 2021</a>
<a href="#">99.2</a>	<a href="#">Press Release, dated February 17, 2021</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

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

Y-MABS THERAPEUTICS, INC.

Date: February 19, 2021

By: /s/ Thomas Gad

\_\_\_\_\_  
Thomas Gad

Founder, Chairman, President and Head of Business Development &  
Strategy

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Y-MABS THERAPEUTICS, INC.

(a Delaware corporation)

2,439,025 Shares of Common Stock

UNDERWRITING AGREEMENT

Dated: February 17, 2021

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Y-MABS THERAPEUTICS, INC.

(a Delaware corporation)

2,439,025 Shares of Common Stock

**UNDERWRITING AGREEMENT**

February 17, 2021

J.P. Morgan Securities LLC  
Morgan Stanley & Co. LLC  
BofA Securities, Inc.

as Representatives of the several Underwriters

c/o J.P. Morgan Securities LLC  
383 Madison Avenue  
New York, New York 10179

c/o Morgan Stanley & Co. LLC  
1585 Broadway  
New York, New York 10036

c/o BofA Securities, Inc.  
One Bryant Park  
New York, New York 10036

Ladies and Gentlemen:

Y-mAbs Therapeutics, Inc., a Delaware corporation (the “Company”), confirms its agreement with J.P. Morgan Securities LLC (“J.P. Morgan”), Morgan Stanley & Co. LLC (“Morgan Stanley”), BofA Securities, Inc. (“BofA”) and each of the other Underwriters named in Schedule A hereto (collectively, the “Underwriters,” which term shall also include any underwriter substituted as hereinafter provided in Section 10 hereof), for whom J.P. Morgan, Morgan Stanley and BofA are acting as representatives (in such capacity, the “Representatives”), with respect to (i) the sale by the Company and the purchase by the Underwriters, acting severally and not jointly, of the respective numbers of shares of Common Stock, par value \$0.0001 per share, of the Company (“Common Stock”) set forth in Schedule A hereto and (ii) the grant by the Company to the Underwriters, acting severally and not jointly, of the option described in Section 2(b) hereof to purchase all or any part of 365,853 additional shares of Common Stock. The aforesaid 2,439,025 shares of Common Stock (the “Initial Securities”) to be purchased by the Underwriters and all or any part of the 365,853 shares of Common Stock subject to the option described in Section 2(b) hereof (the “Option Securities”) are herein called, collectively, the “Securities.”

The Company understands that the Underwriters propose to make a public offering of the Securities as soon as the Representatives deem advisable after this Agreement has been executed and delivered.

The Company has prepared and filed with the Securities and Exchange Commission (the “Commission”) a shelf registration statement on Form S-3 (No. 333-234034), covering the public offering and sale of certain securities, including the Securities, under the Securities Act of 1933, as amended (the “1933 Act”) and the rules and regulations promulgated thereunder (the “1933 Act Regulations”), which shelf registration statement has been declared effective by the Commission. Such registration statement, as of any time, means such registration statement as amended by any post-effective amendments thereto at such time, including the exhibits and any schedules thereto at such time, the documents incorporated or deemed to be incorporated by reference therein at such time pursuant to Item 12 of Form S-3 under the 1933 Act and the documents otherwise deemed to be a part thereof as of such time pursuant to Rule 430B under the 1933 Act Regulations (“Rule 430B”), and is referred to herein as the “Registration Statement,” provided, however, that the “Registration Statement” without reference to a time means such registration statement as amended by any post-effective amendments thereto as of the time of the first contract of sale for the Securities, which time shall be considered the “new effective date” of such registration statement with respect to the Securities within the meaning of paragraph (f)(2) of Rule 430B, including the exhibits and schedules thereto as of such time, the documents incorporated or deemed incorporated by reference therein at such time pursuant to Item 12 of Form S-3 under the 1933 Act and the documents otherwise deemed to be a part thereof as of such time pursuant to Rule 430B. Any registration statement filed pursuant to Rule 462(b) of the 1933 Act Regulations is herein called the “Rule 462(b) Registration Statement” and, after such filing, the term “Registration Statement” shall include the Rule 462(b) Registration Statement. Each preliminary prospectus used in connection with the offering of the Securities, including the documents incorporated or deemed to be incorporated by reference therein pursuant to Item 12 of Form S-3 under the 1933 Act, are collectively referred to herein as a “preliminary prospectus.” Promptly after execution and delivery of this Agreement, the Company will prepare and file a final prospectus relating to the Securities in accordance with the provisions of Rule 424(b) under the 1933 Act Regulations (“Rule 424(b”). The final prospectus, in the form first furnished or made available to the Underwriters for use in connection with the offering of the Securities, including the documents incorporated or deemed to be incorporated by reference therein pursuant to Item 12 of Form S-3 under the 1933 Act, are collectively referred to herein as the “Prospectus.” For purposes of this Agreement, all references to the Registration Statement, any preliminary prospectus, the Prospectus or any amendment or supplement to any of the foregoing shall be deemed to include the copy filed with the Commission pursuant to its Electronic Data Gathering, Analysis and Retrieval system (or any successor system) (“EDGAR”).

As used in this Agreement:

“Applicable Time” means 5:20 P.M., New York City time, on February 17, 2021 or such other time as agreed by the Company and the Representatives.

“General Disclosure Package” means any Issuer General Use Free Writing Prospectuses issued at or prior to the Applicable Time, the most recent preliminary prospectus (including any documents incorporated by reference therein) that is distributed to investors prior to the Applicable Time and the information included on Schedule B-1 hereto, all considered together.

“Issuer Free Writing Prospectus” means any “issuer free writing prospectus,” as defined in Rule 433 of the 1933 Act Regulations (“Rule 433”), including without limitation any “free writing prospectus” (as defined in Rule 405 of the 1933 Act Regulations (“Rule 405”)) relating to the Securities that is (i) required to be filed with the Commission by the Company, (ii) a “road show that is a written communication” within the meaning of Rule 433(d)(8)(i), whether or not required to be filed with the Commission, or (iii) exempt from filing with the Commission pursuant to Rule 433(d)(5)(i) because it contains a description of the Securities or of the offering that does not reflect the final terms, in each case in the form filed or required to be filed with the Commission or, if not required to be filed, in the form retained in the Company’s records pursuant to Rule 433(g).

“Issuer General Use Free Writing Prospectus” means any Issuer Free Writing Prospectus that is intended for general distribution to prospective investors, as evidenced by its being specified in Schedule B-2 hereto.

“Issuer Limited Use Free Writing Prospectus” means any Issuer Free Writing Prospectus that is not an Issuer General Use Free Writing Prospectus.

“Testing-the-Waters Communication” means any oral or written communication with potential investors undertaken in reliance on Section 5(d) of, or Rule 163B under, the 1933 Act.

“Written Testing-the-Waters Communication” means any Testing-the-Waters Communication that is a written communication within the meaning of Rule 405 under the 1933 Act.

All references in this Agreement to financial statements and schedules and other information which is “contained,” “included” or “stated” (or other references of like import) in the Registration Statement, any preliminary prospectus or the Prospectus shall be deemed to include all such financial statements and schedules and other information incorporated or deemed incorporated by reference in the Registration Statement, any preliminary prospectus or the Prospectus, as the case may be, prior to the execution and delivery of this Agreement; and all references in this Agreement to amendments or supplements to the Registration Statement, any preliminary prospectus or the Prospectus shall be deemed to include the filing of any document under the Securities Exchange Act of 1934, as amended (the “1934 Act”) and the rules and regulations promulgated thereunder (the “1934 Regulations”), incorporated or deemed to be incorporated by reference in the Registration Statement, such preliminary prospectus or the Prospectus, as the case may be, at or after the execution and delivery of this Agreement.

#### SECTION 1. Representations and Warranties.

(a) *Representations and Warranties by the Company.* The Company represents and warrants to each Underwriter as of the date hereof, the Applicable Time, the Closing Time (as defined below) and any Date of Delivery (as defined below), and agrees with each Underwriter, as follows:

(i) Registration Statement and Prospectuses. The Company meets the requirements for use of Form S-3 under the 1933 Act for primary offerings. Each of the Registration Statement and any post-effective amendment thereto has become effective under the 1933 Act. No stop order suspending the effectiveness of the Registration Statement or any post-effective amendment thereto has been issued under the 1933 Act, no order preventing or suspending the use of any preliminary prospectus or the Prospectus has been issued and no proceedings for any of those purposes have been instituted or are pending or, to the Company’s knowledge, contemplated by the Commission. The Company has complied with each request (if any) from the Commission for additional information.

Each of the Registration Statement and any post-effective amendment thereto, at the time of its effectiveness and at each deemed effective date with respect to the Underwriters pursuant to Rule 430B(f)(2) under the 1933 Act Regulations, complied and will comply in all material respects with the requirements of the 1933 Act and the 1933 Act Regulations. Each preliminary prospectus, the Prospectus and any amendment or supplement thereto, at the time each was filed with the Commission, complied and will comply in all material respects with the requirements of the 1933 Act and the 1933 Act Regulations. Each preliminary prospectus and the Prospectus delivered to the Underwriters for use in connection with this offering was or will be identical to the electronically transmitted copies thereof filed with the Commission pursuant to EDGAR, except to the extent permitted by Regulation S-T.



The documents incorporated or deemed to be incorporated by reference in the Registration Statement and the Prospectus, when they became effective or at the time they were or hereafter are filed with the Commission, complied and will comply in all material respects with the requirements of the 1934 Act and the 1934 Act Regulations.

(ii) Accurate Disclosure. Neither the Registration Statement nor any amendment thereto, at its effective time, on the date hereof, at the Closing Time or at any Date of Delivery, contained, contains or will contain an untrue statement of a material fact or omitted, omits or will omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading. At the Applicable Time, at the Closing Time and any Date of Delivery, neither of (A) the General Disclosure Package nor (B) any individual Issuer Limited Use Free Writing Prospectus, when considered together with the General Disclosure Package, included, includes or will include an untrue statement of a material fact or omitted, omits or will omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading. Neither the Prospectus nor any amendment or supplement thereto, as of its issue date, at the time of any filing with the Commission pursuant to Rule 424(b), at the Closing Time or at any Date of Delivery, included, includes or will include an untrue statement of a material fact or omitted, omits or will omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading. The documents incorporated or deemed to be incorporated by reference in the Registration Statement, the General Disclosure Package and the Prospectus, at the time the Registration Statement became effective or when such documents incorporated by reference were filed with the Commission, as the case may be, when read together with the other information in the Registration Statement, the General Disclosure Package or the Prospectus, as the case may be, did not and will not include an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading.

The representations and warranties in this subsection shall not apply to statements in or omissions from the Registration Statement (or any amendment thereto), the General Disclosure Package or the Prospectus (or any amendment or supplement thereto) made in reliance upon and in conformity with written information furnished to the Company by any Underwriter through the Representatives expressly for use therein. For purposes of this Agreement, the only information so furnished shall be the information in the (i) third paragraph of text under the caption “Underwriters” in the Prospectus concerning the terms of the offering by the Underwriters; (ii) the eleventh, twelfth and thirteenth paragraph of text under the caption “Underwriters” in the Prospectus concerning stabilization and overallotments by the Underwriters; and (iii) the fifteenth paragraph of text under the caption “Underwriters” in the Prospectus concerning the availability of the prospectus in electronic format, the allocation of shares of common stock to online brokerage account holders, and Internet distributions (collectively, the “Underwriter Information”).

(iii) Issuer Free Writing Prospectuses. No Issuer Free Writing Prospectus conflicts or will conflict with the information contained in the Registration Statement or the Prospectus, including any document incorporated by reference therein, and any preliminary or other prospectus deemed to be a part thereof that has not been superseded or modified. The representations and warranties in this subsection shall not apply to statements in or omissions from any Issuer Free Writing Prospectus made in reliance upon and in conformity with the Underwriter Information.

(iv) Testing-the-Waters Materials. The Company (A) has not engaged in any Testing-the-Waters Communication other than Testing-the-Waters Communications with the consent of the Representatives with entities that are qualified institutional buyers within the meaning of Rule 144A under the 1933 Act or institutions that are accredited investors within the meaning of Rule 501 under the 1933 Act or, in the case of Testing-the-Waters Communications made in reliance on Rule 163B, with entities that the Company reasonably believes are qualified institutional buyers or institutions that are accredited investors within the meaning of Rule 163B under the 1933 Act and (B) has not authorized anyone other than the Representatives to engage in Testing-the-Waters Communications. The Company reconfirms that the Representatives have been authorized to act on its behalf in undertaking Testing-the-Waters Communications. The Company has not distributed any Written Testing-the-Waters Communications.

(v) Company Not Ineligible Issuer. At the time of filing the Registration Statement and any post-effective amendment thereto, at the earliest time thereafter that the Company or another offering participant made a *bona fide* offer (within the meaning of Rule 164(h)(2) of the 1933 Act Regulations) of the Securities and at the date hereof, the Company was not and is not an “ineligible issuer,” as defined in Rule 405, without taking account of any determination by the Commission pursuant to Rule 405 that it is not necessary that the Company be considered an ineligible issuer.

(vi) [Reserved].

(vii) Independent Accountants. The accountants who certified the financial statements and supporting schedules included in the Registration Statement, the General Disclosure Package and the Prospectus are independent public accountants as required by the 1933 Act, the 1933 Act Regulations, the 1934 Act, the 1934 Act Regulations and the Public Company Accounting Oversight Board.

(viii) Financial Statements. The financial statements included or incorporated by reference in the Registration Statement, the General Disclosure Package and the Prospectus, together with the related schedules and notes, present fairly in all material respects the financial position of the Company and its subsidiaries at the dates indicated and the statement of operations, stockholders’ equity and cash flows of the Company and its subsidiaries for the periods specified; said financial statements have been prepared in conformity with U.S. generally accepted accounting principles (“GAAP”) applied on a consistent basis throughout the periods involved. The supporting schedules, if any, present fairly, in all material respects, in accordance with GAAP the information required to be stated therein. The selected financial data and the summary financial information included in the Registration Statement, the General Disclosure Package and the Prospectus present fairly, in all material respects, the information shown therein and have been compiled on a basis consistent with that of the audited financial statements included therein. Except as included therein, no historical or pro forma financial statements or supporting schedules are required to be included or incorporated by reference in the Registration Statement, the General Disclosure Package or the Prospectus under the 1933 Act or the 1933 Act Regulations, the 1934 Act or the 1934 Act Regulations. The interactive data in eXtensible Business Reporting Language incorporated by reference in the Registration Statement, the General Disclosure Package and the Prospectus fairly presents the information called for in all material respects and has been prepared in accordance with the Commission's rules and guidelines applicable thereto.

(ix) No Material Adverse Change in Business. Except as otherwise stated therein, since the respective dates as of which information is given in the Registration Statement, the General Disclosure Package or the Prospectus, (A) there has been no material adverse change in the condition, financial or otherwise, or in the earnings, business affairs or business prospects of the Company and its subsidiaries considered as one enterprise, whether or not arising in the ordinary course of business (a “Material Adverse Effect”), (B) there have been no transactions entered into by the Company or its subsidiaries, other than those in the ordinary course of business, which are material with respect to the Company and its subsidiaries considered as one enterprise and (C) there has been no dividend or distribution of any kind declared, paid or made by the Company on any class of its capital stock.

(x) Good Standing of the Company. The Company has been duly organized and is validly existing as a corporation in good standing under the laws of the State of Delaware and has corporate power and authority to own, lease and operate its properties and to conduct its business as described in the Registration Statement, the General Disclosure Package and the Prospectus and to enter into and perform its obligations under this Agreement; and the Company is duly qualified as a foreign corporation to transact business and is in good standing in each other jurisdiction in which such qualification is required (or such equivalent concept to the extent it exists under the laws of such jurisdiction), whether by reason of the ownership or leasing of property or the conduct of business, except where the failure to so qualify or to be in good standing would not reasonably be expected to result in a Material Adverse Effect.

(xi) Good Standing of the Subsidiaries. The subsidiaries have been duly organized and is validly existing in good standing under the laws of the jurisdiction of its incorporation or organization (or such equivalent concept to the extent it exists under the laws of such jurisdiction), has corporate or similar power and authority to own, lease and operate its properties and to conduct its business as described in the Registration Statement, the General Disclosure Package and the Prospectus and is duly qualified to transact business and is in good standing in each jurisdiction in which such qualification is required (or such equivalent concept to the extent it exists under the laws of such jurisdiction), whether by reason of the ownership or leasing of property or the conduct of business, except where the failure to so qualify or to be in good standing would not reasonably be expected to result in a Material Adverse Effect. Except as otherwise disclosed in the Registration Statement, the General Disclosure Package and the Prospectus, all of the issued and outstanding capital stock of the subsidiaries have been duly authorized and validly issued, is fully paid and non-assessable and is owned directly by the Company, free and clear of any security interest, mortgage, pledge, lien, encumbrance, claim or equity. None of the outstanding shares of capital stock of the subsidiaries were issued in violation of the preemptive or similar rights of any securityholder of the subsidiaries. The only significant subsidiary of the Company (as such term is defined in Rule 1-02 of Regulation S-X) is Y-mAbs Therapeutics A/S, a company organized under the laws of the Kingdom of Denmark.

(xii) Capitalization. The authorized, issued and outstanding shares of capital stock of the Company are as set forth in the Registration Statement, the General Disclosure Package and the Prospectus in the column entitled “Actual” under the caption “Capitalization” (except for subsequent issuances, if any, pursuant to this Agreement, pursuant to reservations, agreements or employee benefit or equity incentive plans referred to in the Registration Statement, the General Disclosure Package and the Prospectus or pursuant to the exercise of convertible securities or options referred to in the Registration Statement, the General Disclosure Package and the Prospectus). The outstanding shares of capital stock of the Company have been duly authorized and validly issued and are fully paid and non-assessable. None of the outstanding shares of capital stock of the Company were issued in violation of the preemptive or other similar rights of any securityholder of the Company.

(xiii) Authorization of Agreement. This Agreement has been duly authorized, executed and delivered by the Company.

(xiv) Authorization and Description of Securities. The Securities to be purchased by the Underwriters from the Company have been duly authorized for issuance and sale to the Underwriters pursuant to this Agreement and, when issued and delivered by the Company pursuant to this Agreement against payment of the consideration set forth herein, will be validly issued and fully paid and non-assessable; and the issuance of the Securities is not subject to the preemptive or other similar rights of any securityholder of the Company. The Common Stock conforms, in all material respects, to all statements relating thereto contained in the Registration Statement, the General Disclosure Package and the Prospectus and such description conforms, in all material respects, to the rights set forth in the instruments defining the same. No holder of Securities will be subject to personal liability by reason of being such a holder.

(xv) Registration Rights. There are no persons with registration rights or other similar rights to have any securities registered for sale pursuant to the Registration Statement or otherwise registered for sale or sold by the Company under the 1933 Act pursuant to this Agreement, other than those rights that have been disclosed in the Registration Statement, the General Disclosure Package and the Prospectus and have been waived.

(xvi) Absence of Violations, Defaults and Conflicts. Neither the Company nor any of its subsidiaries is (A) in violation of its charter, by-laws or similar organizational document, (B) in default in the performance or observance of any obligation, agreement, covenant or condition contained in any contract, indenture, mortgage, deed of trust, loan or credit agreement, note, lease or other agreement or instrument to which the Company or its subsidiaries are a party or by which it or any of them may be bound or to which any of the properties or assets of the Company or its subsidiaries are subject (collectively, "Agreements and Instruments"), except for such defaults that would not, singly or in the aggregate, result in a Material Adverse Effect, or (C) in violation of any law, statute, rule, regulation, judgment, order, writ or decree of any arbitrator, court, governmental body, regulatory body, administrative agency or other authority, body or agency having jurisdiction over the Company or its subsidiaries or any of their respective properties, assets or operations (each, a "Governmental Entity"), except for such violations that would not, singly or in the aggregate, result in a Material Adverse Effect. The execution, delivery and performance of this Agreement and the consummation of the transactions contemplated herein (including the issuance and sale of the Securities and the use of the proceeds from the sale of the Securities as described in the Registration Statement, the General Disclosure Package and the Prospectus under the caption "Use of Proceeds") and compliance by the Company with its obligations hereunder have been duly authorized by all necessary corporate action and do not and will not, whether with or without the giving of notice or passage of time or both, conflict with or constitute a breach of, or default or Repayment Event (as defined below) under, or result in the creation or imposition of any lien, charge or encumbrance upon any properties or assets of the Company or its subsidiaries pursuant to, the Agreements and Instruments (except for such conflicts, breaches, defaults or Repayment Events or liens, charges or encumbrances that would not, singly or in the aggregate, result in a Material Adverse Effect), nor will such action result in (i) any violation of the provisions of the charter, by-laws or similar organizational document of the Company or its subsidiaries or (ii) any law, statute, rule, regulation, judgment, order, writ or decree of any Governmental Entity, except in the case of clause (ii) for such violations that would not, singly or in the aggregate, result in a Material Adverse Effect. As used herein, a "Repayment Event" means any event or condition which gives the holder of any note, debenture or other evidence of indebtedness (or any person acting on such holder's behalf) the right to require the repurchase, redemption or repayment of all or a portion of such indebtedness by the Company or its subsidiaries.

(xvii) Absence of Labor Dispute. No labor dispute with the employees of the Company or any of its subsidiaries exists or, to the knowledge of the Company, is imminent, and the Company is not aware of any existing or imminent labor disturbance by the employees of any of its or its subsidiaries' principal suppliers, manufacturers, collaborators, customers, or contractors, which, in either case, would reasonably be expected to result in a Material Adverse Effect.

(xviii) Absence of Proceedings. Except as disclosed in the Registration Statement, the General Disclosure Package and the Prospectus, there is no action, suit, proceeding, inquiry or investigation before or brought by any Governmental Entity (including, without limitation, any action, suit, proceeding, inquiry or investigation before or brought by the U.S. Food and Drug Administration (the "FDA")) now pending or, to the knowledge of the Company, threatened, against or affecting the Company or its subsidiaries, which would result in a Material Adverse Effect, or which would materially and adversely affect their respective properties or assets or the consummation of the transactions contemplated in this Agreement or the performance by the Company of its obligations hereunder; and the aggregate of all pending legal or governmental proceedings to which the Company or its subsidiaries are a party or of which any of their respective properties or assets is the subject which are not described in the Registration Statement, the General Disclosure Package and the Prospectus, including ordinary routine litigation incidental to the business, would not result in a Material Adverse Effect.

(xix) Accuracy of Exhibits. There are no contracts or documents which are required under the 1933 Act Regulations to be described in the Registration Statement, the General Disclosure Package or the Prospectus or to be filed as exhibits to the Registration Statement which have not been so described in all material respects and filed as required.

(xx) Absence of Further Requirements. No filing with, or authorization, approval, consent, license, order, registration, qualification or decree of, any Governmental Entity is necessary or required for the performance by the Company of its obligations hereunder, in connection with the offering, issuance or sale of the Securities hereunder or the consummation of the transactions contemplated by this Agreement, except (A) such as have been already obtained or as may be required under the 1933 Act, the 1933 Act Regulations, the rules of the Nasdaq Global Select Market, state securities laws or the rules of the Financial Industry Regulatory Authority, Inc. ("FINRA") and (B) such as which the failure to obtain would not, singly or in the aggregate, materially impair the power or ability of the Company to perform its obligations under this Agreement or consummate the transactions contemplated thereby.

(xxi) Possession of Licenses and Permits. Each of the Company and its subsidiaries possess such permits, licenses, approvals, consents and other authorizations (collectively, "Governmental Licenses") issued by the appropriate Governmental Entities necessary to conduct its business as currently being conducted by each of them (including, without limitation, all such permits, licenses, approvals, consents and other authorizations required by the FDA, the European Medicines Agency, or any other federal, state, local or foreign agencies or bodies engaged in the regulation of clinical trials, pharmaceuticals, biologics or biohazardous substances or materials), except where the failure so to possess would not, singly or in the aggregate, result in a Material Adverse Effect. The Company and its subsidiaries are in compliance with the terms and conditions of all Governmental Licenses, except where the failure so to comply would not, singly or in the aggregate, result in a Material Adverse Effect. All of the Governmental Licenses are valid and in full force and effect, except when the invalidity of such Governmental Licenses or the failure of such Governmental Licenses to be in full force and effect would not, singly or in the aggregate, result in a Material Adverse Effect. Neither the Company nor its subsidiaries have received any notice of proceedings relating to the revocation or modification of any Governmental Licenses which, singly or in the aggregate, if the subject of an unfavorable decision, ruling or finding, would result in a Material Adverse Effect. The Company and its subsidiaries (i) are, and at all times have been, in compliance with all statutes, rules and regulations applicable to the ownership, testing, development, manufacture, packaging, processing, use, distribution, storage, import, export or disposal of any product manufactured or distributed by the Company or its subsidiaries ("Applicable Laws"), except where such noncompliance would not, singly or in the aggregate, result in a Material Adverse Effect; and (ii) have not received any FDA Form 483, written notice of adverse finding, warning letter, untitled letter or other correspondence or written notice from any court or arbitrator or governmental or regulatory authority alleging or asserting noncompliance with (x) any Applicable Laws or (y) any licenses, exemptions, certificates, approvals, clearances, authorizations, permits and supplements or amendments thereto required by any such Applicable Laws, except where being in contravention of any of the foregoing representations or warranties, singly or in the aggregate, would not have a Material Adverse Effect.

(xxii) Title to Property. The Company and its subsidiaries do not own any real property. The Company and its subsidiaries have good title to all other properties (excluding for the purpose of this section Intellectual Property (as defined below), as to which the Company's and its subsidiaries' representations and warranties are set forth in Section 1(a)(xxiii) below) owned by them, in each case, free and clear of all mortgages, pledges, liens, security interests, claims, restrictions or encumbrances of any kind except such as (A) are described in the Registration Statement, the General Disclosure Package and the Prospectus or (B) do not, singly or in the aggregate, materially adversely affect the value of such property and do not materially interfere with the use made and proposed to be made of such property by the Company or its subsidiaries; and all of the leases and subleases material to the business of the Company and its subsidiaries, considered as one enterprise, and under which the Company or its subsidiaries hold properties described in the Registration Statement, the General Disclosure Package or the Prospectus, are in full force and effect, and neither the Company nor its subsidiaries have any notice of any material claim of any sort that has been asserted by anyone adverse to the rights of the Company or its subsidiaries under any of the leases or subleases mentioned above, or affecting or questioning the rights of the Company or its subsidiaries to the continued possession of the leased or subleased premises under any such lease or sublease, except to the extent that where such failure to be in effect or such claim or adverse effect on the Company's rights would not reasonably be expected to result in a Material Adverse Effect.

(xxiii) Possession of Intellectual Property. Except as disclosed in the Registration Statement, the General Disclosure Package and the Prospectus, the Company and its subsidiaries own or possess, have license to, or can acquire on reasonable terms, all patents, patent rights, inventions, copyrights, technology, data, know-how (including trade secrets and other unpatented and/or unpatentable proprietary or confidential information, systems or procedures), trademarks, service marks, trade names and/or other intellectual property (collectively, “Intellectual Property”) necessary to carry on their business as currently being conducted by them, and to the knowledge of the Company, as disclosed in the Registration Statement, the General Disclosure Package and the Prospectus, and neither the Company nor its subsidiaries have received any notice or is otherwise aware of (A) any infringement or violation of any Intellectual Property rights of others by the Company or its subsidiaries, or (B) any facts or circumstances which could render any Intellectual Property owned by or exclusively licensed to the Company or its subsidiaries (such Intellectual Property, the “Company Intellectual Property”) invalid or unenforceable, and which infringement, or violation (if the subject of any unfavorable decision, ruling or finding) or invalidity or unenforceability, singly or in the aggregate, would result in a Material Adverse Effect. To the knowledge of the Company, there is no infringement or violation of any Company Intellectual Property by third parties. The Company and its subsidiaries have complied in all material respects with the terms of each agreement pursuant to which Intellectual Property has been licensed to the Company or its subsidiaries, and, to the Company’s knowledge, all such agreements are in full force and effect. All Company Intellectual Property have been duly maintained and are in full force and effect and, to the Company’s knowledge, there are no material defects in any of the Company Intellectual Property. Each person who is or was an employee or contractor of the Company or its subsidiaries and who is or was involved in the creation or development of any Intellectual Property for or on behalf of the Company or its subsidiaries have signed an agreement containing an assignment to the Company or its subsidiaries of such person’s rights in and to such Intellectual Property and, to the Company’s knowledge, no employee of the Company or its subsidiaries is in or has ever been in violation of any term of any agreement or covenant to or with a former employer where the basis of such violation relates to such employee’s employment with the Company or its subsidiaries or actions undertaken by the employee while employed with the Company or its subsidiaries. The Company has taken reasonable steps to protect, maintain and safeguard its rights and licenses under Intellectual Property owned by or licensed to the Company, including the execution of appropriate nondisclosure and confidentiality agreements.

(xxiv) Environmental Laws. Except as described in the Registration Statement, the General Disclosure Package and the Prospectus or would not, singly or in the aggregate, reasonably be expected to result in a Material Adverse Effect, (A) neither the Company nor its subsidiaries are in violation of any applicable federal, state, local or foreign statute, law, rule, regulation, ordinance, code, or rule of common law or any judicial or administrative interpretation thereof, including any judicial or administrative order, consent, decree or judgment, relating to pollution or protection of human health, the environment (including, without limitation, ambient air, surface water, groundwater, land surface or subsurface strata) or wildlife, including, without limitation, laws and regulations relating to the release or threatened release of hazardous chemicals, pollutants, contaminants, hazardous wastes, toxic substances, hazardous substances, petroleum or petroleum products, asbestos-containing materials or toxic mold (collectively, “Hazardous Materials”) or to the manufacture, processing, distribution, use, treatment, storage, disposal, transport or handling of Hazardous Materials (collectively, “Environmental Laws”), (B) the Company and its subsidiaries have all permits, authorizations and approvals required under any applicable Environmental Laws for the operation of their business as currently being conducted or the occupancy of their real property and are each in compliance with their requirements, (C) there are no pending or, to the knowledge of the Company, threatened administrative, regulatory or judicial actions, suits, demands, demand letters, claims, liens, notices of noncompliance or violation, investigations or proceedings relating to any Environmental Law against the Company or its subsidiaries and (D) there are no events or circumstances that would reasonably be expected to form the basis of an order for clean-up or remediation, or an action, suit or proceeding by any private party or Governmental Entity, against or affecting the Company or its subsidiaries relating to Hazardous Materials or any Environmental Laws.

(xxv) Accounting Controls and Disclosure Controls. The Company and its subsidiaries maintain effective internal control over financial reporting (as defined under Rules 13-a15 and 15d-15 under the 1934 Act Regulations) and a system of internal accounting controls sufficient to provide reasonable assurances that (A) transactions are executed in accordance with management's general or specific authorization; (B) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain accountability for assets; (C) access to assets is permitted only in accordance with management's general or specific authorization; (D) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences; and (E) the interactive data in eXtensible Business Reporting Language incorporated by reference in the Registration Statement, the General Disclosure Package and the Prospectus fairly presents the information called for in all material respects and is prepared in accordance with the Commission's rules and guidelines applicable thereto. Except as described in the Registration Statement, the General Disclosure Package and the Prospectus, since the end of the Company's most recent audited fiscal year, there has been (1) no material weakness in the Company's internal control over financial reporting (whether or not remediated) and (2) no change in the Company's internal control over financial reporting that has materially adversely affected, or is reasonably likely to materially adversely affect, the Company's internal control over financial reporting. The Company and its subsidiaries maintain an effective system of disclosure controls and procedures (as defined in Rule 13a-15 and Rule 15d-15 under the 1934 Act Regulations) that are designed to ensure that information required to be disclosed by the Company in the reports that it files or submits under the 1934 Act is recorded, processed, summarized and reported, within the time periods specified in the Commission's rules and forms, and is accumulated and communicated to the Company's management, including its principal executive officer or officers and principal financial officer or officers, as appropriate, to allow timely decisions regarding disclosure.

(xxvi) Compliance with the Sarbanes-Oxley Act. The Company has taken all necessary actions to ensure that it is in compliance with all provisions of the Sarbanes-Oxley Act of 2002 and all rules and regulations promulgated thereunder or implementing the provisions thereof (the "Sarbanes-Oxley Act") that are in effect and with which the Company is required to comply.

(xxvii) Payment of Taxes. All United States federal, state, local and non-U.S. income tax returns of the Company and its subsidiaries required by applicable law to be filed have been filed and all taxes shown by such returns or otherwise assessed, which are due and payable, have been paid, except assessments being contested in good faith and as to which adequate reserves have been established. No assessments in connection with the United States federal, state, local and non-U.S. income tax returns of the Company and its subsidiaries through the fiscal year ended December 31, 2020 have been made against the Company or its subsidiaries. The Company and its subsidiaries have timely filed all other tax returns that are required to have been filed by them pursuant to applicable foreign, state, local or other law except insofar as the failure to file such returns would not result in a Material Adverse Effect, and has paid all taxes due pursuant to such returns or pursuant to any assessment received by the Company and its subsidiaries, except for such taxes, if any, as are being contested in good faith and as to which adequate reserves have been established in accordance with GAAP by the Company. The charges, accruals and reserves on the books of the Company and its subsidiaries in respect of any income tax liability for any years not finally determined are adequate to meet any assessments or re-assessments for additional income tax for any years not finally determined, except to the extent of any inadequacy that would not result in a Material Adverse Effect.

(xxviii) Insurance. The Company and its subsidiaries carry or are entitled to the benefits of insurance, with financially sound and reputable insurers, in such amounts and covering such risks as the Company reasonably believes is generally maintained by companies of established repute engaged in the same or similar business, and all such insurance is in full force and effect. The Company has no reason to believe that it or its subsidiaries will not be able (A) to renew its existing insurance coverage as and when such policies expire or (B) to obtain comparable coverage from similar institutions as may be necessary or appropriate to conduct its business as now conducted and at a cost that would not reasonably be expected to result in a Material Adverse Effect. Neither of the Company nor its subsidiaries have been denied any insurance coverage which it has sought or for which it has applied.

(xxix) Investment Company Act. The Company is not required, and upon the issuance and sale of the Securities as herein contemplated and the application of the net proceeds therefrom as described in the Registration Statement, the General Disclosure Package and the Prospectus will not be required, to register as an “investment company” under the Investment Company Act of 1940, as amended (the “1940 Act”).

(xxx) Absence of Manipulation. Neither the Company nor any of its subsidiaries, nor to the Company’s knowledge any of their respective directors, officers or affiliates, has taken, nor will any of the foregoing take, directly or indirectly, any action which is designed, or would reasonably be expected, to cause or result in, or which constitutes, the stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of the Securities or to result in a violation of Regulation M under the 1934 Act.

(xxxi) Foreign Corrupt Practices Act. None of the Company, its subsidiaries or, to the knowledge of the Company, any director, officer, agent, employee, affiliate or other person acting on behalf of the Company or its subsidiaries are aware of or has taken any action, directly or indirectly, that would result in a violation by such persons of the Foreign Corrupt Practices Act of 1977, as amended, and the rules and regulations thereunder (the “FCPA”), including, without limitation, making use of the mails or any means or instrumentality of interstate commerce corruptly in furtherance of an offer, payment, promise to pay or authorization of the payment of any money, or other property, gift, promise to give, or authorization of the giving of anything of value to any “foreign official” (as such term is defined in the FCPA) or any foreign political party or official thereof or any candidate for foreign political office, in contravention of the FCPA and the Company and, to the knowledge of the Company, its controlled affiliates have conducted their businesses in compliance with the FCPA and have instituted and maintain policies and procedures designed to ensure, and which are reasonably expected to continue to ensure, continued compliance therewith.

(xxxii) Money Laundering Laws. The operations of the Company and its subsidiaries are and have been conducted at all times in compliance with applicable financial recordkeeping and reporting requirements of the Currency and Foreign Transactions Reporting Act of 1970, as amended, the applicable money laundering statutes of all applicable jurisdictions, the rules and regulations thereunder and any related or similar applicable rules, regulations or guidelines, issued, administered or enforced by any Governmental Entity (collectively, the “Money Laundering Laws”); and no action, suit or proceeding by or before any Governmental Entity involving the Company or its subsidiaries with respect to the Money Laundering Laws is pending or, to the knowledge of the Company, threatened.



(xxxiii) OFAC. None of the Company, its subsidiaries or, to the knowledge of the Company and its subsidiaries, any director, officer, agent, employee, affiliate or representative of the Company or its subsidiaries is a Person currently the subject or target of any sanctions administered or enforced by the United States Government, including, without limitation, the U.S. Department of the Treasury's Office of Foreign Assets Control ("OFAC"), the United Nations Security Council, the European Union, Her Majesty's Treasury, or other relevant sanctions authority (collectively, "Sanctions"), nor is the Company located, organized or resident in a country or territory that is the subject of Sanctions; and the Company will not directly or indirectly use the proceeds of the sale of the Securities, or lend, contribute or otherwise make available such proceeds to its subsidiaries, joint venture partners or other Person, to fund any activities of or business with any Person, or in any country or territory, that, at the time of such funding, is the subject of Sanctions or in any other manner that will result in a violation by any Person (including any Person participating in the transaction, whether as underwriter, advisor, investor or otherwise) of Sanctions.

(xxxiv) Lending Relationship. The Company (i) does not have any material lending or other relationship with any bank or lending affiliate of any Underwriter and (ii) does not intend to use any of the proceeds from the sale of the Securities to repay any outstanding debt owed to any affiliate of any Underwriter.

(xxxv) Statistical and Market-Related Data. Any statistical and market-related data included in the Registration Statement, the General Disclosure Package or the Prospectus are based on or derived from sources that the Company believes, after reasonable inquiry, to be reliable and accurate in all material respects, and to the extent legally or contractually required, the Company has obtained the written consent to the use of such data from such sources.

(xxxvi) Regulatory Matters. Except as described in the Registration Statement, the General Disclosure Package and the Prospectus, and except as would not, individually or in the aggregate, have or may reasonably be expected to have a Material Adverse Effect: (i) neither the Company nor its subsidiaries have received any written notice of adverse filing, warning letter, untitled letter or other correspondence or notice from the FDA or other relevant regulatory authorities, or any other court or arbitrator or federal, state, local or foreign governmental or regulatory authority, alleging or asserting material noncompliance with the Federal Food, Drug and Cosmetic Act (21 U.S.C. § 301 et seq.) (the "FFDCA"), or similar state, federal or foreign law or regulation; (ii) the Company and its subsidiaries and, to the Company's knowledge, their respective directors, officers, employees or agents, are and since April 30, 2015 have been in compliance in all material respects with applicable health care laws, including without limitation, the FFDCA and the federal Anti-Kickback Statute (42 U.S.C. § 1320a-7b(b)), the Civil Monetary Penalties Law (42 U.S.C. § 1320a-7a), the U.S. Civil False Claims Act (31 U.S.C. § 3729 et seq.), 18 U.S.C. §§ 286 and 287, and the health care fraud criminal provisions under the U.S. Health Insurance Portability and Accountability Act of 1996 ("HIPAA") (42 U.S.C. Section 1320d et seq.), the exclusion laws (42 U.S.C. § 1320a-7), HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (42 U.S.C. § 17921 et seq.), the U.S. Controlled Substances Act (21 U.S.C. Section 801 et seq.), each as amended, and the regulations promulgated thereunder; and all other comparable local, state, federal, national, supranational and foreign laws, and the regulations promulgated thereunder (collectively, "Health Care Laws"); (iii) neither the Company nor its subsidiaries received written notice of any ongoing claim, action, suit, proceeding, hearing, enforcement, investigation, arbitration or other action from any U.S. or non-U.S. federal, national, state, local or other governmental or regulatory authority, governmental or regulatory agency or body, court, arbitrator or self-regulatory organization (each, a "Governmental Authority") or third party alleging that any product operation or activity is in violation of any Health Care Laws or has any knowledge that any such Governmental Authority or third party is considering any such claim, litigation, arbitration, action, suit, investigation or proceeding; (iv) neither the Company nor its subsidiaries have received written notice that any Governmental Authority has taken, is taking or intends to take action to limit, suspend, modify or revoke any applicable permit or has any knowledge that any such Governmental Authority is considering such action; (v) the Company and its subsidiaries have filed, obtained, maintained or submitted all reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments as required by any Health Care Laws and that all such reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments were complete, correct and not misleading on the date filed (or were corrected or supplemented by a subsequent submission); (vi) neither the Company nor its subsidiaries have, either voluntarily or involuntarily, initiated, conducted, or issued or caused to be initiated, conducted or issued, any recall, field notifications, field corrections, market withdrawals or replacements, warnings, safety alert, post-sale warning, "dear doctor" letter, investigator notices or other notice or action relating to the alleged lack of safety, efficacy or regulatory compliance of any product or any alleged product defect or violation and, to the Company's knowledge, no third party has initiated or conducted any such notice or action ("Safety Notices") and there are no facts that would be reasonably likely to result in (x) a Safety Notice with respect to the Company's or its subsidiaries' products, or (y) a material change in labeling of any of the Company's or its subsidiaries' products or (z) a termination or suspension of marketing or testing of any of the Company's or its subsidiaries' products; and (vii) neither the Company nor its subsidiaries or any of their respective directors, officers, employees or agents is or has been debarred, suspended or excluded, or has been convicted of any crime or engaged in any conduct that would result in a debarment, suspension or exclusion from any federal or state government health care program.

(xxxvii) Clinical Trials. The clinical and preclinical trials conducted by, on behalf of or sponsored by the Company or its subsidiaries, or in which the Company or its subsidiaries have participated, that are described in the Registration Statement, the General Disclosure Package and the Prospectus, or the results of which are referred to in the Registration Statement, the General Disclosure Package and the Prospectus, as applicable, were, and if still pending are, being conducted in all material respects in accordance with standard medical and scientific research procedures, standard industry practice, current Good Clinical Practices and Good Laboratory Practices and all applicable rules and regulations of the FDA and comparable drug regulatory agencies outside of the United States to which they are subject (collectively, the “Regulatory Authorities”); the descriptions in the Registration Statement, the General Disclosure Package or the Prospectus of the results of such studies and tests are accurate and complete in all material respects and fairly present the data derived from such trials; the Company has no knowledge of any other trials not described in the Registration Statement, the General Disclosure Package and the Prospectus, the results of which are inconsistent with or reasonably call into question the results described or referred to in the Registration Statement, the General Disclosure Package and the Prospectus; the Company and its subsidiaries have operated at all times and are currently in compliance in all material respects with all applicable statutes, rules and regulations of the Regulatory Authorities; neither the Company nor its subsidiaries have received any written notices, correspondence or other communications from the Regulatory Authorities or any other governmental agency requiring or threatening the termination, material modification or suspension of any clinical or preclinical trials that are described in the Registration Statement, the General Disclosure Package and the Prospectus or the results of which are referred to in the Registration Statement, the General Disclosure Package and the Prospectus, other than ordinary course communications with respect to modifications in connection with the design and implementation of such trials, and, to the Company’s knowledge, there are no reasonable grounds for the same.

(xxxviii) Health Care Products Manufacturing. The manufacture of the Company's and its subsidiaries' products by or on behalf of the Company and its subsidiaries is being conducted in compliance in all material respects with all applicable Health Care Laws, including, without limitation, the FDA's current good manufacturing practice regulations at 21 CFR Part 820, and, to the extent applicable, the respective counterparts thereof promulgated by governmental authorities in countries outside the United States. Neither the Company nor its subsidiaries have had any manufacturing site (whether Company-owned, subsidiary-owned or that of a third party manufacturer for the Company's or its subsidiaries' products) subject to a Governmental Authority (including FDA or EMA) shutdown or import or export prohibition, nor received any FDA, EMA or other Governmental Authority "warning letters," or "untitled letters" alleging or asserting material noncompliance with any applicable Health Care Laws, requests to make material changes to the Company's or its subsidiaries' products, processes or operations, or similar correspondence or notice from the FDA, EMA or other Governmental Authority alleging or asserting material noncompliance with any applicable Health Care Laws, other than those that have been satisfactorily addressed and/or closed with the FDA, EMA or other Governmental Authority. To the knowledge of the Company, neither the FDA, EMA or any other Governmental Authority is considering such action.

(xxxix) No Rated Securities. Neither the Company nor its subsidiaries have any debt securities or preferred stock that are rated by any "nationally recognized statistical rating organization" (as defined in Section 3(a)(62) of the 1934 Act).

(xl) Cybersecurity. (A) There has been no security breach or incident, unauthorized access or disclosure, or other compromise of or relating to the Company's or its subsidiaries' information technology and computer systems, networks, hardware, software, data and databases (including the data and information of their respective customers, employees, suppliers, vendors and any third party data maintained, processed or stored by the Company and its subsidiaries, and any such data processed or stored by third parties on behalf of the Company and its subsidiaries), equipment or technology (collectively, "IT Systems and Data"); (B) neither the Company nor its subsidiaries have been notified of, and each of them have no knowledge of any event or condition that could result in, any security breach or incident, unauthorized access or disclosure or other compromise to their IT Systems and Data and (C) the Company and its subsidiaries have implemented appropriate controls, policies, procedures, and technological safeguards to maintain and protect the integrity, continuous operation, redundancy and security of their IT Systems and Data reasonably consistent with industry standards and practices, or as required by applicable regulatory standards. The Company and its subsidiaries are presently in material compliance with all applicable laws or statutes and all judgments, orders, rules and regulations of any court or arbitrator or governmental or regulatory authority, internal policies and contractual obligations relating to the privacy and security of IT Systems and Data and to the protection of such IT Systems and Data from unauthorized use, access, misappropriation or modification.

(xli) ERISA Compliance. The Company, its subsidiaries, and any of their "ERISA Affiliates" (defined below) each represents, warrants and covenants that they: (i) do not maintain sponsor or participate in any tax-qualified defined benefit plan, as defined under Section 3(3) of the Employee Retirement Income Security Act of 1974, as amended, and the regulations and published interpretations thereunder (collectively, "ERISA"); (ii) do not sponsor or participate in a plan subject to any other federal, state or local law that is materially similar to Section 406 of ERISA or Section 4975 of the Internal Revenue Code of 1986, as amended, and the regulations and published interpretations thereunder (the "Code"); (iii) do not maintain, sponsor or participate in an "Employee Benefit Plan," as defined under Section 3(3) or ERISA, that is intended to be qualified under Section 401(a) of the Code; and (iv) are otherwise in compliance in all material respects with ERISA. "ERISA Affiliate" means, with respect to the Company or its subsidiaries, any member of any group of organizations described in Sections 414(b), (c), (m) or (o) of the Code of which the Company or its subsidiaries are a member. Neither the Company, its subsidiaries nor any of their ERISA Affiliates has incurred or reasonably expects to incur any liability under (i) Title IV of ERISA with respect to termination of, or withdrawal from, any Employee Benefit Plan or (ii) Sections 412 and 430, 4971, 4975 or 4980B of the Code.

(b) *Officer's Certificates.* Any certificate signed by any officer of the Company or its subsidiaries delivered to the Representatives or to counsel for the Underwriters shall be deemed a representation and warranty by the Company (and not by such officer in his or her personal capacity) to each Underwriter as to the matters covered thereby.

SECTION 2. Sale and Delivery to Underwriters; Closing.

(a) *Initial Securities.* On the basis of the representations and warranties herein contained and subject to the terms and conditions herein set forth, the Company agrees to sell to each Underwriter, severally and not jointly, and each Underwriter, severally and not jointly, agrees to purchase from the Company, at the price per share set forth in Schedule A, that number of Initial Securities set forth in Schedule A opposite the name of such Underwriter, plus any additional number of Initial Securities which such Underwriter may become obligated to purchase pursuant to the provisions of Section 10 hereof, subject, in each case, to such adjustments among the Underwriters as the Representatives in their sole discretion shall make to eliminate any sales or purchases of fractional shares.

(b) *Option Securities.* In addition, on the basis of the representations and warranties herein contained and subject to the terms and conditions herein set forth, the Company hereby grants an option to the Underwriters, severally and not jointly, to purchase up to an additional 365,853 shares of Common Stock, at the price per share set forth in Schedule A, less an amount per share equal to any dividends or distributions declared by the Company and payable on the Initial Securities but not payable on the Option Securities. The option hereby granted may be exercised for 30 days after the date hereof and may be exercised in whole or in part at any time from time to time upon notice by the Representatives to the Company setting forth the number of Option Securities as to which the several Underwriters are then exercising the option and the time and date of payment and delivery for such Option Securities. Any such time and date of delivery (a "Date of Delivery") shall be determined by the Representatives, but shall not be later than seven full business days after the exercise of said option, nor in any event prior to the Closing Time. If the option is exercised as to all or any portion of the Option Securities, each of the Underwriters, acting severally and not jointly, will purchase that proportion of the total number of Option Securities then being purchased which the number of Initial Securities set forth in Schedule A opposite the name of such Underwriter bears to the total number of Initial Securities, subject, in each case, to such adjustments as the Representatives in their sole discretion shall make to eliminate any sales or purchases of fractional shares.

(c) *Payment.* Payment of the purchase price for, and delivery of certificates or security entitlements for, the Initial Securities shall be made at the offices of Shearman & Sterling LLP, 599 Lexington Avenue, New York, New York 10022, or at such other place as shall be agreed upon by the Representatives and the Company, at 9:00 A.M. (New York City time) on the second (third, if the pricing occurs after 4:30 P.M. (New York City time) on any given day) business day after the date hereof (unless postponed in accordance with the provisions of Section 10), or such other time not later than ten business days after such date as shall be agreed upon by the Representatives and the Company (such time and date of payment and delivery being herein called "Closing Time").

In addition, in the event that any or all of the Option Securities are purchased by the Underwriters, payment of the purchase price for, and delivery of certificates or security entitlements for, such Option Securities shall be made at the above-mentioned offices, or at such other place as shall be agreed upon by the Representatives and the Company, on each Date of Delivery as specified in the notice from the Representatives to the Company.

Payment shall be made to the Company by wire transfer of immediately available funds to a bank account designated by the Company against delivery to the Representatives for the respective accounts of the Underwriters of certificates or security entitlements for the Securities to be purchased by them. It is understood that each Underwriter has authorized the Representatives, for its account, to accept delivery of, receipt for, and make payment of the purchase price for, the Initial Securities and the Option Securities, if any, which it has agreed to purchase. Each of the Representatives, individually and not as representative of the Underwriters, may (but shall not be obligated to) make payment of the purchase price for the Initial Securities or the Option Securities, if any, to be purchased by any Underwriter whose funds have not been received by the Closing Time or the relevant Date of Delivery, as the case may be, but such payment shall not relieve such Underwriter from its obligations hereunder.

SECTION 3. Covenants of the Company. The Company covenants with each Underwriter as follows:

(a) *Compliance with Securities Regulations and Commission Requests*. The Company, subject to Section 3(b), will comply with the requirements of Rule 430B, and will notify the Representatives promptly, and confirm the notice in writing, (i) when any post-effective amendment to the Registration Statement shall become effective or any amendment or supplement to the Prospectus shall have been filed, (ii) of the receipt of any comments from the Commission, (iii) of any request by the Commission for any amendment to the Registration Statement or any amendment or supplement to the Prospectus, including any document incorporated by reference therein or for additional information, (iv) of the issuance by the Commission of any stop order suspending the effectiveness of the Registration Statement or any post-effective amendment or of any order preventing or suspending the use of any preliminary prospectus or the Prospectus, or of the suspension of the qualification of the Securities for offering or sale in any jurisdiction, or of the initiation or threatening of any proceedings for any of such purposes or of any examination pursuant to Section 8(d) or 8(e) of the 1933 Act concerning the Registration Statement and (v) if the Company becomes the subject of a proceeding under Section 8A of the 1933 Act in connection with the offering of the Securities. The Company will effect all filings required under Rule 424(b), in the manner and within the time period required by Rule 424(b) (without reliance on Rule 424(b)(8)), and will take such steps as it deems necessary to ascertain promptly whether the form of prospectus transmitted for filing under Rule 424(b) was received for filing by the Commission and, in the event that it was not, it will promptly file such prospectus. The Company will make every reasonable effort to prevent the issuance of any stop order, prevention or suspension and, if any such order is issued, to obtain the lifting thereof as soon as reasonably practicable.

(b) *Continued Compliance with Securities Laws.* The Company will comply with the 1933 Act, the 1933 Act Regulations, the 1934 Act and the 1934 Act Regulations so as to permit the completion of the distribution of the Securities as contemplated in this Agreement and in the Registration Statement, the General Disclosure Package and the Prospectus. If at any time when a prospectus relating to the Securities is (or, but for the exception afforded by Rule 172 of the 1933 Act Regulations (“Rule 172”), would be) required by the 1933 Act to be delivered in connection with sales of the Securities, any event shall occur or condition shall exist as a result of which it is necessary, in the opinion of counsel for the Underwriters or for the Company, to (i) amend the Registration Statement in order that the Registration Statement will not include an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading, (ii) amend or supplement the General Disclosure Package or the Prospectus in order that the General Disclosure Package or the Prospectus, as the case may be, will not include any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein not misleading in the light of the circumstances existing at the time it is delivered to a purchaser or (iii) amend the Registration Statement or amend or supplement the General Disclosure Package or the Prospectus, as the case may be, in order to comply with the requirements of the 1933 Act or the 1933 Act Regulations, the Company will promptly (A) give the Representatives notice of such event, (B) prepare any amendment or supplement as may be necessary to correct such statement or omission or to make the Registration Statement, the General Disclosure Package or the Prospectus comply with such requirements and, a reasonable amount of time prior to any proposed filing or use, furnish the Representatives with copies of any such amendment or supplement and (C) file with the Commission any such amendment or supplement; provided that the Company shall not file or use any such amendment or supplement to which the Representatives or counsel for the Underwriters shall reasonably object. The Company will furnish to the Underwriters such number of copies of such amendment or supplement as the Underwriters may reasonably request. The Company has given the Representatives notice of any filings made pursuant to the 1934 Act or 1934 Act Regulations within 48 hours prior to the Applicable Time; the Company will give the Representatives notice of its intention to make any such filing from the Applicable Time to the Closing Time and will furnish the Representatives with copies of any such documents a reasonable amount of time prior to such proposed filing, as the case may be, and will not file or use any such document to which the Representatives or counsel for the Underwriters shall reasonably object.

(c) *Delivery of Registration Statements.* The Company has furnished or will deliver to the Representatives and counsel for the Underwriters, without charge, signed copies of the Registration Statement as originally filed and each amendment thereto (including exhibits filed therewith or incorporated by reference therein and documents incorporated or deemed to be incorporated by reference therein) and signed copies of all consents and certificates of experts, and will also deliver to the Representatives, without charge, a conformed copy of the Registration Statement as originally filed and each amendment thereto (without exhibits) for each of the Underwriters. The copies of the Registration Statement and each amendment thereto furnished to the Underwriters will be identical to the electronically transmitted copies thereof filed with the Commission pursuant to EDGAR, except to the extent permitted by Regulation S-T.

(d) *Delivery of Prospectuses.* The Company has delivered to each Underwriter, without charge, as many copies of each preliminary prospectus as such Underwriter reasonably requested, and the Company hereby consents to the use of such copies for purposes permitted by the 1933 Act. The Company will furnish to each Underwriter, without charge, during the period when a prospectus relating to the Securities is (or, but for the exception afforded by Rule 172, would be) required to be delivered under the 1933 Act, such number of copies of the Prospectus (as amended or supplemented) as such Underwriter may reasonably request. The Prospectus and any amendments or supplements thereto furnished to the Underwriters will be identical to the electronically transmitted copies thereof filed with the Commission pursuant to EDGAR, except to the extent permitted by Regulation S-T.

(e) *Certification Regarding Beneficial Owners.* The Company will deliver to the Representatives, on the date of execution of this Agreement, a properly completed and executed Certification Regarding Beneficial Owners of Legal Entity Customers, together with copies of identifying documentation, and the Company will undertake to provide such additional supporting documentation as the Representatives may reasonably request in connection with the verification of the foregoing certification.

(f) *Blue Sky Qualifications.* The Company will use its best efforts, in cooperation with the Underwriters, to qualify the Securities for offering and sale under the applicable securities laws of such states and other jurisdictions (domestic or foreign) as the Representatives may reasonably designate and to maintain such qualifications in effect so long as required to complete the distribution of the Securities; provided, however, that the Company shall not be obligated to file any general consent to service of process or to qualify as a foreign corporation or as a dealer in securities in any jurisdiction in which it is not so qualified or to subject itself to taxation in respect of doing business in any jurisdiction in which it is not otherwise so subject.

(g) *Rule 158.* The Company will timely file such reports pursuant to the 1934 Act as are necessary in order to make generally available to its securityholders as soon as practicable an earnings statement for the purposes of, and to provide to the Underwriters the benefits contemplated by, the last paragraph of Section 11(a) of the 1933 Act.

(h) *Use of Proceeds.* The Company will use the net proceeds received by it from the sale of the Securities in the manner specified in the Registration Statement, the General Disclosure Package and the Prospectus under "Use of Proceeds."

(i) *Listing.* The Company will use its best efforts to effect and its reasonable best efforts to maintain the listing of the Securities on the Nasdaq Global Select Market.

(j) *Restriction on Sale of Securities.* During a period of 90 days from the date of the Prospectus, the Company will not, without the prior written consent of the Representatives, (i) directly or indirectly, offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise transfer or dispose of any shares of Common Stock or any securities convertible into or exercisable or exchangeable for Common Stock or file any registration statement under the 1933 Act with respect to any of the foregoing or (ii) enter into any swap or any other agreement or any transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of the Common Stock, whether any such swap or transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or other securities, in cash or otherwise or (iii) publicly disclose the intention to do any of the foregoing described in clauses (i) or (ii). The foregoing sentence shall not apply to (A) the Securities to be sold hereunder, (B) any shares of Common Stock issued by the Company upon the exercise of an option or warrant or the conversion of a security outstanding on the date hereof and referred to in the Registration Statement, the General Disclosure Package and the Prospectus, (C) any shares of Common Stock issued or options to purchase Common Stock granted pursuant to existing employee benefit or equity incentive plans of the Company referred to in the Registration Statement, the General Disclosure Package and the Prospectus, (D) any shares of Common Stock issued pursuant to any non-employee director stock plan or dividend reinvestment plan referred to in the Registration Statement, the General Disclosure Package and the Prospectus, (E) the filing by the Company of a registration statement on Form S-8 or any successor form thereto with respect to the registration of securities to be offered under any employee benefit or equity incentive plans of the Company referred to in the Registration Statement, the General Disclosure Package and the Prospectus, or (F) shares of Common Stock or other securities issued in connection with a transaction that includes a commercial relationship (including strategic alliances, commercial lending relationships, joint ventures and strategic acquisitions), provided that (x) the aggregate number of shares issued pursuant to this clause (F) shall not exceed 5.0% of the total number of outstanding shares of Common Stock immediately following the issuance and sale of the Securities at the Closing Time pursuant hereto and (y) the recipient of any such shares of Common Stock or securities issued pursuant to this clause (F) during the 90-day restricted period described above shall enter into an agreement substantially in the form of Exhibit B hereto.

(k) *Reporting Requirements.* The Company, during the period when a Prospectus relating to the Securities is (or, but for the exception afforded by Rule 172, would be) required to be delivered under the 1933 Act, will file all documents required to be filed with the Commission pursuant to the 1934 Act within the time periods required by the 1934 Act and 1934 Act Regulations. Additionally, the Company shall report the use of proceeds from the issuance of the Securities as may be required under Rule 463 under the 1933 Act.

(l) *Issuer Free Writing Prospectuses.* The Company agrees that, unless it obtains the prior written consent of the Representatives, it will not make any offer relating to the Securities that would constitute an Issuer Free Writing Prospectus or that would otherwise constitute a “free writing prospectus,” or a portion thereof, required to be filed by the Company with the Commission or retained by the Company under Rule 433; provided that the Representatives will be deemed to have consented to the Issuer Free Writing Prospectuses listed on Schedule B-2 hereto and any “road show that is a written communication” within the meaning of Rule 433(d)(8)(i) that has been reviewed by the Representatives. The Company represents that it has treated or agrees that it will treat each such free writing prospectus consented to, or deemed consented to, by the Representatives as an “issuer free writing prospectus,” as defined in Rule 433, and that it has complied and will comply with the applicable requirements of Rule 433 with respect thereto, including timely filing with the Commission where required, legending and record keeping. If at any time following issuance of an Issuer Free Writing Prospectus there occurred or occurs an event or development as a result of which such Issuer Free Writing Prospectus conflicted or would conflict with the information contained in the Registration Statement, any preliminary prospectus or the Prospectus or included or would include an untrue statement of a material fact or omitted or would omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances existing at that subsequent time, not misleading, the Company will promptly notify the Representatives and will promptly amend or supplement, at its own expense, such Issuer Free Writing Prospectus to eliminate or correct such conflict, untrue statement or omission. Each Underwriter represents that it has not made, and agrees that, without the prior consent of the Company, it will not make any offer relating to the Securities that would constitute a “free writing prospectus” required to be filed by the Company with the Commission or retained by the Company under Rule 433 of the 1933 Act Regulations; provided that the Company will be deemed to have consented to any “road show” that has been reviewed and approved by the Company and is a written communication within the meaning of Rule 433(d)(8)(i) of the 1933 Act Regulations.

#### SECTION 4. Payment of Expenses.

(a) *Expenses.* The Company will pay or cause to be paid all expenses incident to the performance of its obligations under this Agreement, including (i) the preparation, printing and filing of the Registration Statement (including financial statements and exhibits) as originally filed and each amendment thereto, (ii) the preparation, printing and delivery to the Underwriters of copies of each preliminary prospectus, each Issuer Free Writing Prospectus and the Prospectus and any amendments or supplements thereto and any costs associated with electronic delivery of any of the foregoing by the Underwriters to investors, (iii) the preparation, issuance and delivery of the certificates or security entitlements for the Securities to the Underwriters, including any stock or other transfer taxes and any stamp or other duties payable upon the sale, issuance or delivery of the Securities to the Underwriters, (iv) the fees and disbursements of the Company’s counsel, accountants and other advisors, (v) the qualification of the Securities under securities laws in accordance with the provisions of Section 3(f) hereof, including filing fees and the reasonable and documented fees and disbursements of counsel for the Underwriters in connection therewith and in connection with the preparation of the Blue Sky Survey and any supplement thereto, (vi) the fees and expenses of any transfer agent or registrar for the Securities, (vii) the costs and expenses of the Company relating to investor presentations on any “road show” (as defined in Rule 433(h)(4) under the 1933 Act) undertaken in connection with the marketing of the Securities, including without limitation, expenses associated with the production of road show slides and graphics, reasonable and documented fees and expenses of any consultants engaged with the prior authorization of the Company in connection with the road show presentations, travel and lodging expenses of the representatives and officers of the Company and any such consultants (which, for the avoidance of doubt, does not include the Underwriters or their representatives), and the cost of transportation in connection with the road show, (viii) the filing fees incident to, and the reasonable and documented fees and disbursements of counsel to the Underwriters in connection with, the review by FINRA of the terms of the sale of the Securities; provided that the amount payable by the Company for counsel pursuant to the foregoing clause (v) and clause (viii) together shall not exceed \$35,000 in the aggregate, (ix) the fees and expenses incurred in connection with the listing of the Securities on the Nasdaq Global Select Market and (x) the costs and expenses (including, without limitation, any damages or other amounts payable in connection with legal or contractual liability) associated with the reforming of any contracts for sale of the Securities made by the Underwriters caused by a breach of the representation contained in the third sentence of Section 1(a)(ii).



(b) *Termination of Agreement.* If this Agreement is terminated by the Representatives in accordance with the provisions of Section 5, Section 9(a)(i) or (iii) or Section 10 hereof, the Company shall reimburse the Underwriters for all of their reasonable and documented out-of-pocket expenses, including the reasonable and documented fees and disbursements of counsel for the Underwriters. For the avoidance of doubt, if this Agreement is terminated pursuant to Section 10, the Company shall only be required to reimburse the reasonable and documented out-of-pocket expenses (including the reasonable and documented fees and disbursements of counsel for the Underwriters) of the Underwriters that have not failed to purchase the Securities that they have agreed to purchase hereunder.

SECTION 5. Conditions of Underwriters' Obligations. The obligations of the several Underwriters hereunder are subject to the accuracy of the representations and warranties of the Company contained herein or in certificates of any officer of the Company or its subsidiaries delivered pursuant to the provisions hereof, to the performance by the Company of its covenants and other obligations hereunder, and to the following further conditions:

(a) *Effectiveness of Registration Statement.* The Registration Statement, including any Rule 462(b) Registration Statement, has become effective and, at the Closing Time, no stop order suspending the effectiveness of the Registration Statement or any post-effective amendment thereto has been issued under the 1933 Act, no order preventing or suspending the use of any preliminary prospectus or the Prospectus has been issued and no proceedings for any of those purposes have been instituted or are pending or, to the Company's knowledge, contemplated by the Commission; and the Company has complied with each request (if any) from the Commission for additional information.

(b) *Opinion of Counsel for Company.* At the Closing Time, the Representatives shall have received the opinion and negative assurance letter, dated the Closing Time, of Duane Morris LLP, counsel for the Company, in form and substance satisfactory to counsel for the Underwriters, together with signed or reproduced copies of such letter for each of the other Underwriters.

(c) *Opinion of Counsel for Danish Subsidiary.* At the Closing Time, the Representatives shall have received the opinion, dated the Closing Time, of Mazanti-Andersen, counsel for Y-mAbs Therapeutics A/S, in form and substance satisfactory to counsel for the Underwriters, together with signed or reproduced copies of such letter for each of the other Underwriters.

(d) *Opinions of Patent and Regulatory Counsel for Company.* At the Closing Time, the Representatives shall have received the opinions, dated the Closing Time, of (i) Nelson Mullins Riley & Scarborough LLP, U.S. patent counsel for the Company, (ii) Valua ApS, European patent counsel for the Company, and (iii) Cooley LLP, regulatory counsel for the Company, respectively, in form and substance satisfactory to counsel for the Underwriters, together with signed or reproduced copies of such letter for each of the other Underwriters.

(e) *Opinion of Counsel for Underwriters.* At the Closing Time, the Representatives shall have received the opinion and negative assurance letter, dated the Closing Time, of Shearman & Sterling LLP, counsel for the Underwriters, in form and substance satisfactory to the Underwriters.

(f) *Officers' Certificate.* At the Closing Time, there shall not have been, since the date hereof or since the respective dates as of which information is given in the Registration Statement, the General Disclosure Package or the Prospectus, any material adverse change in the condition, financial or otherwise, or in the earnings, business affairs or business prospects of the Company and its subsidiaries considered as one enterprise, whether or not arising in the ordinary course of business, and the Representatives shall have received a certificate of the Chief Executive Officer or the President of the Company and of the chief financial or chief accounting officer of the Company, in their respective capacities as such officers only, dated the Closing Time, to the effect that (i) there has been no such material adverse change, (ii) the representations and warranties of the Company in this Agreement are true and correct with the same force and effect as though expressly made at and as of the Closing Time, (iii) the Company has complied with all agreements and satisfied all conditions on its part to be performed or satisfied at or prior to the Closing Time, and (iv) no stop order suspending the effectiveness of the Registration Statement under the 1933 Act has been issued, no order preventing or suspending the use of any preliminary prospectus or the Prospectus has been issued and no proceedings for any of those purposes have been instituted or are pending or, to their knowledge, contemplated.

(g) *Accountant's Comfort Letter.* At the time of the execution of this Agreement, the Representatives shall have received from PricewaterhouseCoopers LLP a letter, dated such date, in form and substance satisfactory to the Representatives, together with signed or reproduced copies of such letter for each of the other Underwriters containing statements and information of the type ordinarily included in accountants' "comfort letters" to underwriters with respect to the financial statements and certain financial information contained in the Registration Statement, the General Disclosure Package and the Prospectus.

(h) *Bring-down Comfort Letter.* At the Closing Time, the Representatives shall have received from PricewaterhouseCoopers LLP a letter, dated as of the Closing Time, to the effect that they reaffirm the statements made in the letter furnished pursuant to subsection (f) of this Section, except that the specified date referred to shall be a date not more than three business days prior to the Closing Time.

(i) *Approval of Listing.* At the Closing Time, the Securities shall have been approved for listing on the Nasdaq Global Select Market, subject only to official notice of issuance.

(j) *No Objection.* FINRA has confirmed that it has not raised any objection with respect to the fairness and reasonableness of the underwriting terms and arrangements relating to the offering of the Securities.

(k) *Lock-up Agreements.* At the date of this Agreement, the Representatives shall have received an agreement substantially in the form of Exhibit B hereto signed by the Persons listed on Schedule C hereto.

(l) *Chief Financial Officer's Certificate.* At the time of the execution of this Agreement and at the Closing Time, the Representatives shall have received from the chief financial officer of the Company a certificate in a form reasonably satisfactory to the Underwriters.

(m) *Conditions to Purchase of Option Securities.* In the event that the Underwriters exercise their option provided in Section 2(b) hereof to purchase all or any portion of the Option Securities, the representations and warranties of the Company contained herein and the statements in any certificates furnished by the Company and its subsidiaries hereunder shall be true and correct as of each Date of Delivery and, at the relevant Date of Delivery, the Representatives shall have received:

(i) Officers' Certificate. A certificate, dated such Date of Delivery, of the Chief Executive Officer or the President of the Company and of the chief financial or chief accounting officer of the Company confirming that the certificate delivered at the Closing Time pursuant to Section 5(e) hereof remains true and correct as of such Date of Delivery.

(ii) Opinion of Counsel for Company. If requested by the Representatives, the opinion of Duane Morris LLP, counsel for the Company, in form and substance satisfactory to counsel for the Underwriters, dated such Date of Delivery, relating to the Option Securities to be purchased on such Date of Delivery and otherwise to the same effect as the opinion required by Section 5(b) hereof.

(iii) Opinion of Counsel for Danish Subsidiary. If requested by the Representatives, the opinion of Mazanti-Andersen, counsel for Y-mAbs Therapeutics A/S, in form and substance satisfactory to counsel for the Underwriters, dated such Date of Delivery, relating to the Option Securities to be purchased on such Date of Delivery and otherwise to the same effect as the opinion required by Section 5(c) hereof.

(iv) Opinions of Patent and Regulatory Counsel for Company. If requested by the Representatives, the opinions of (i) Nelson Mullins Riley & Scarborough LLP, U.S. patent counsel for the Company, in form and substance satisfactory to counsel for the Underwriters, (ii) Valua ApS, European patent counsel for the Company, in form and substance satisfactory to counsel for the Underwriters, and (iii) Cooley LLP, regulatory counsel for the Company, in form and substance satisfactory to counsel for the Underwriters, each dated such Date of Delivery, relating to the Option Securities to be purchased on such Date of Delivery and otherwise to the same effect as the respective opinions required by Section 5(d) hereof.

(v) Opinion of Counsel for Underwriters. If requested by the Representatives, the opinion of Shearman & Sterling LLP, counsel for the Underwriters, dated such Date of Delivery, relating to the Option Securities to be purchased on such Date of Delivery and otherwise to the same effect as the opinion required by Section 5(e) hereof.

(vi) Bring-down Comfort Letter. If requested by the Representatives, a letter from PricewaterhouseCoopers LLP, in form and substance satisfactory to the Representatives and dated such Date of Delivery, substantially in the same form and substance as the letter furnished to the Representatives pursuant to Section 5(h) hereof, except that the "specified date" in the letter furnished pursuant to this paragraph shall be a date not more than three business days prior to such Date of Delivery.

(vii) Chief Financial Officer's Certificate. A certificate from the chief financial officer of the Company, in form and substance satisfactory to the Underwriters and dated such Date of Delivery, substantially in the same form and substance as the letter furnished to the Representatives pursuant to Section 5(l) hereof.

(n) *Additional Documents.* At the Closing Time and at each Date of Delivery (if any) counsel for the Underwriters shall have been furnished with such documents and opinions as they may reasonably require for the purpose of enabling them to pass upon the issuance and sale of the Securities as herein contemplated, or in order to evidence the accuracy of any of the representations or warranties, or the fulfillment of any of the conditions, herein contained; and all proceedings taken by the Company in connection with the issuance and sale of the Securities as herein contemplated shall be satisfactory in form and substance to the Representatives and counsel for the Underwriters.

(o) *Termination of Agreement.* If any condition specified in this Section shall not have been fulfilled when and as required to be fulfilled, this Agreement, or, in the case of any condition to the purchase of Option Securities on a Date of Delivery which is after the Closing Time, the obligations of the several Underwriters to purchase the relevant Option Securities, may be terminated by the Representatives by notice to the Company at any time at or prior to Closing Time or such Date of Delivery, as the case may be, and such termination shall be without liability of any party to any other party except as provided in Section 4 and except that Sections 1, 6, 7, 8, 14, 15, 16 and 17 shall survive any such termination and remain in full force and effect.

SECTION 6. Indemnification.

(a) *Indemnification of Underwriters.* The Company agrees to indemnify and hold harmless each Underwriter, its officers, directors, employees, affiliates (as such term is defined in Rule 501(b) under the 1933 Act (each, an “Affiliate”)), its selling agents and each person, if any, who controls any Underwriter within the meaning of Section 15 of the 1933 Act or Section 20 of the 1934 Act as follows:

(i) against any and all loss, liability, claim, damage and expense whatsoever, as incurred, arising out of any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement (or any amendment thereto), including any information deemed to be a part thereof pursuant to Rule 430B, or the omission or alleged omission therefrom of a material fact required to be stated therein or necessary to make the statements therein not misleading or arising out of any untrue statement or alleged untrue statement of a material fact included (A) in any preliminary prospectus, any Issuer Free Writing Prospectus, the General Disclosure Package or the Prospectus (or any amendment or supplement thereto), or (B) in any materials or information provided to investors by, or with the approval of, the Company in connection with the marketing of the offering of the Securities (“Marketing Materials”), including any road show or investor presentations made to investors by the Company (whether in person or electronically), or the omission or alleged omission in any preliminary prospectus, Issuer Free Writing Prospectus, Prospectus or in any Marketing Materials of a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading;

(ii) against any and all loss, liability, claim, damage and expense whatsoever, as incurred, to the extent of the aggregate amount paid in settlement of any litigation, or any investigation or proceeding by any governmental agency or body, commenced or threatened, or of any claim whatsoever based upon any such untrue statement or omission, or any such alleged untrue statement or omission; provided that (subject to Section 6(d) below) any such settlement is effected with the written consent of the Company;

(iii) against any and all reasonably documented expense whatsoever, as incurred (including the fees and disbursements of one counsel (in addition to any local counsel) chosen by the Representatives), reasonably incurred in investigating, preparing or defending against any litigation, or any investigation or proceeding by any governmental agency or body, commenced or threatened, or any claim whatsoever based upon any such untrue statement or omission, or any such alleged untrue statement or omission, to the extent that any such expense is not paid under (i) or (ii) above;

provided, however, that this indemnity agreement shall not apply to any loss, liability, claim, damage or expense to the extent arising out of any untrue statement or omission or alleged untrue statement or omission made in the Registration Statement (or any amendment thereto), including any information deemed to be a part thereof pursuant to Rule 430B, the General Disclosure Package or the Prospectus (or any amendment or supplement thereto) in reliance upon and in conformity with the Underwriter Information.

(b) *Indemnification of Company, Directors and Officers.* Each Underwriter severally agrees to indemnify and hold harmless the Company, its directors, each of its officers who signed the Registration Statement, and each person, if any, who controls the Company within the meaning of Section 15 of the 1933 Act or Section 20 of the 1934 Act, against any and all loss, liability, claim, damage and expense described in the indemnity contained in subsection (a) of this Section, as incurred, but only with respect to untrue statements or omissions, or alleged untrue statements or omissions, made in the Registration Statement (or any amendment thereto), including any information deemed to be a part thereof pursuant to Rule 430B, the General Disclosure Package or the Prospectus (or any amendment or supplement thereto) in reliance upon and in conformity with the Underwriter Information.

(c) *Actions against Parties; Notification.* Each indemnified party shall give notice as promptly as reasonably practicable to each indemnifying party of any action commenced against it in respect of which indemnity may be sought hereunder, but failure to so notify an indemnifying party shall not relieve such indemnifying party from any liability hereunder to the extent it is not materially prejudiced as a result thereof and in any event shall not relieve it from any liability which it may have otherwise than on account of this indemnity agreement. In the case of parties indemnified pursuant to Section 6(a) above, counsel to the indemnified parties shall be selected by the Representatives, and, in the case of parties indemnified pursuant to Section 6(b) above, counsel to the indemnified parties shall be selected by the Company. An indemnifying party may participate at its own expense in the defense of any such action; provided, however, that counsel to the indemnifying party shall not (except with the consent of the indemnified party) also be counsel to the indemnified party. In no event shall the indemnifying parties be liable for fees and expenses of more than one counsel (in addition to any local counsel) separate from their own counsel for all indemnified parties in connection with any one action or separate but similar or related actions in the same jurisdiction arising out of the same general allegations or circumstances. No indemnifying party shall, without the prior written consent of the indemnified parties, settle or compromise or consent to the entry of any judgment with respect to any litigation, or any investigation or proceeding by any governmental agency or body, commenced or threatened, or any claim whatsoever in respect of which indemnification or contribution could be sought under this Section 6 or Section 7 hereof (whether or not the indemnified parties are actual or potential parties thereto), unless such settlement, compromise or consent (i) includes an unconditional release of each indemnified party from all liability arising out of such litigation, investigation, proceeding or claim and (ii) does not include a statement as to or an admission of fault, culpability or a failure to act by or on behalf of any indemnified party.

(d) *Settlement without Consent if Failure to Reimburse.* If at any time an indemnified party shall have requested an indemnifying party to reimburse the indemnified party for fees and expenses of counsel, such indemnifying party agrees that it shall be liable for any settlement of the nature contemplated by Section 6(a)(ii) effected without its written consent if (i) such settlement is entered into more than 60 days after receipt by such indemnifying party of the aforesaid request, (ii) such indemnifying party shall have received notice of the terms of such settlement at least 30 days prior to such settlement being entered into and (iii) such indemnifying party shall not have reimbursed such indemnified party in accordance with such request prior to the date of such settlement.

SECTION 7. Contribution. If the indemnification provided for in Section 6 hereof is for any reason unavailable to or insufficient to hold harmless an indemnified party in respect of any losses, liabilities, claims, damages or expenses referred to therein, then each indemnifying party shall contribute to the aggregate amount of such losses, liabilities, claims, damages and expenses incurred by such indemnified party, as incurred, (i) in such proportion as is appropriate to reflect the relative benefits received by the Company, on the one hand, and the Underwriters, on the other hand, from the offering of the Securities pursuant to this Agreement or (ii) if the allocation provided by clause (i) is not permitted by applicable law, in such proportion as is appropriate to reflect not only the relative benefits referred to in clause (i) above but also the relative fault of the Company, on the one hand, and of the Underwriters, on the other hand, in connection with the statements or omissions, which resulted in such losses, liabilities, claims, damages or expenses, as well as any other relevant equitable considerations.

The relative benefits received by the Company, on the one hand, and the Underwriters, on the other hand, in connection with the offering of the Securities pursuant to this Agreement shall be deemed to be in the same respective proportions as the total net proceeds from the offering of the Securities pursuant to this Agreement (before deducting expenses) received by the Company, on the one hand, and the total underwriting discount received by the Underwriters, on the other hand, in each case as set forth on the cover of the Prospectus, bear to the aggregate public offering price of the Securities as set forth on the cover of the Prospectus.

The relative fault of the Company, on the one hand, and the Underwriters, on the other hand, shall be determined by reference to, among other things, whether any such untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact relates to information supplied by the Company or by the Underwriters and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

The Company and the Underwriters agree that it would not be just and equitable if contribution pursuant to this Section 7 were determined by pro rata allocation (even if the Underwriters were treated as one entity for such purpose) or by any other method of allocation which does not take account of the equitable considerations referred to above in this Section 7. The aggregate amount of losses, liabilities, claims, damages and expenses incurred by an indemnified party and referred to above in this Section 7 shall be deemed to include any legal or other expenses reasonably incurred by such indemnified party in investigating, preparing or defending against any litigation, or any investigation or proceeding by any governmental agency or body, commenced or threatened, or any claim whatsoever based upon any such untrue or alleged untrue statement or omission or alleged omission.

Notwithstanding the provisions of this Section 7, no Underwriter shall be required to contribute any amount in excess of the underwriting commissions received by such Underwriter in connection with the Securities underwritten by it and distributed to the public.

No person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the 1933 Act) shall be entitled to contribution from any person who was not guilty of such fraudulent misrepresentation.

For purposes of this Section 7, each person, if any, who controls an Underwriter within the meaning of Section 15 of the 1933 Act or Section 20 of the 1934 Act and each Underwriter's Affiliates and selling agents shall have the same rights to contribution as such Underwriter, and each director of the Company, each officer of the Company who signed the Registration Statement, and each person, if any, who controls the Company within the meaning of Section 15 of the 1933 Act or Section 20 of the 1934 Act shall have the same rights to contribution as the Company. The Underwriters' respective obligations to contribute pursuant to this Section 7 are several in proportion to the number of Initial Securities set forth opposite their respective names in Schedule A hereto and not joint.

SECTION 8. Representations, Warranties and Agreements to Survive. All representations, warranties and agreements contained in this Agreement or in certificates of officers of the Company or its subsidiaries submitted pursuant hereto, shall remain operative and in full force and effect regardless of (i) any investigation made by or on behalf of any Underwriter or its Affiliates or selling agents, any person controlling any Underwriter, its officers or directors or any person controlling the Company and (ii) delivery of and payment for the Securities.

SECTION 9. Termination of Agreement.

(a) *Termination.* The Representatives may terminate this Agreement, by notice to the Company, at any time at or prior to the Closing Time (i) if there has been, in the judgment of the Representatives, since the time of execution of this Agreement or since the respective dates as of which information is given in the Registration Statement, the General Disclosure Package or the Prospectus, any material adverse change in the condition, financial or otherwise, or in the earnings, business affairs or business prospects of the Company and its subsidiaries considered as one enterprise, whether or not arising in the ordinary course of business, or (ii) if there has occurred any material adverse change in the financial markets in the United States or the international financial markets, any outbreak of hostilities or escalation thereof or other calamity or crisis or any change or development involving a prospective change in national or international political, financial or economic conditions, in each case the effect of which is such as to make it, in the judgment of the Representatives, impracticable or inadvisable to proceed with the completion of the offering or to enforce contracts for the sale of the Securities, or (iii) if trading in any securities of the Company has been suspended or materially limited by the Commission or the Nasdaq Global Select Market, or (iv) if trading generally on the NYSE MKT or the New York Stock Exchange or in the Nasdaq Global Select Market has been suspended or materially limited, or minimum or maximum prices for trading have been fixed, or maximum ranges for prices have been required, by any of said exchanges or by order of the Commission, FINRA or any other Governmental Authority, or (v) a material disruption has occurred in commercial banking or securities settlement or clearance services in the United States or with respect to Clearstream or Euroclear systems in Europe, or (vi) if a banking moratorium has been declared by either Federal or New York authorities.

(b) *Liabilities.* If this Agreement is terminated pursuant to this Section, such termination shall be without liability of any party to any other party except as provided in Section 4 hereof, and provided further that Sections 1, 6, 7, 8, 14, 15, 16 and 17 shall survive such termination and remain in full force and effect.

SECTION 10. Default by One or More of the Underwriters. If one or more of the Underwriters shall fail at the Closing Time or a Date of Delivery to purchase the Securities which it or they are obligated to purchase under this Agreement (the "Defaulted Securities"), the Representatives shall have the right, within 24 hours thereafter, to make arrangements for one or more of the non-defaulting Underwriters, or any other underwriters reasonably satisfactory to the Company, to purchase all, but not less than all, of the Defaulted Securities in such amounts as may be agreed upon and upon the terms herein set forth; if, however, the Representatives shall not have completed such arrangements within such 24-hour period, then:

(i) if the number of Defaulted Securities does not exceed 10% of the number of Securities to be purchased on such date, each of the non-defaulting Underwriters shall be obligated, severally and not jointly, to purchase the full amount thereof in the proportions that their respective underwriting obligations hereunder bear to the underwriting obligations of all non-defaulting Underwriters, or

(ii) if the number of Defaulted Securities exceeds 10% of the number of Securities to be purchased on such date, this Agreement or, with respect to any Date of Delivery which occurs after the Closing Time, the obligation of the Underwriters to purchase, and the Company to sell, the Option Securities to be purchased and sold on such Date of Delivery shall terminate without liability on the part of any non-defaulting Underwriter.

No action taken pursuant to this Section shall relieve any defaulting Underwriter from liability in respect of its default.

Any termination of this Agreement pursuant to this Section 10 shall be without liability on the part of the Company except that the Company will continue to be liable for the payment of expenses as set forth in Section 4 hereof and except that the provisions of Section 6 hereof shall not terminate and shall remain in effect.

In the event of any such default which does not result in a termination of this Agreement or, in the case of a Date of Delivery which is after the Closing Time, which does not result in a termination of the obligation of the Underwriters to purchase and the Company to sell the relevant Option Securities, as the case may be, either the (i) Representatives or (ii) the Company shall have the right to postpone the Closing Time or the relevant Date of Delivery, as the case may be, for a period not exceeding seven days in order to effect any required changes in the Registration Statement, the General Disclosure Package or the Prospectus or in any other documents or arrangements. As used herein, the term "Underwriter" includes any person substituted for an Underwriter under this Section 10.

SECTION 11. Notices. All notices and other communications hereunder shall be in writing and shall be deemed to have been duly given if mailed or transmitted by any standard form of telecommunication. Notices to the Underwriters shall be directed to (i) J.P. Morgan at 383 Madison Avenue, New York, New York 10179, fax (212) 622-8358; and (ii) Morgan Stanley at 1585 Broadway, New York, New York 10036, Attention: Equity Syndicate Desk, with a copy to the Legal Department; (iii) BofA at One Bryant Park, New York, New York 10036, attention of Syndicate Department (facsimile: (646) 855-3073), with a copy to ECM Legal (facsimile: (212) 230-8730); with a copy to Shearman & Sterling LLP at 599 Lexington Avenue, New York, NY 10022, attention of Ilir Mujalovic and Harald Halbhuber. Notices to the Company shall be directed to it at Y-mAbs Therapeutics, Inc., 230 Park Avenue, Suite 3350, New York, New York 10169, attention: Thomas Gad, Chairman, with a copy to Duane Morris LLP at 230 Park Avenue, Suite 1130, New York, New York 10169, attention Dwight A. Kinsey.

SECTION 12. No Advisory or Fiduciary Relationship. The Company acknowledges and agrees that (a) the purchase and sale of the Securities pursuant to this Agreement, including the determination of the public offering price of the Securities and any related discounts and commissions, is an arm's-length commercial transaction between the Company, on the one hand, and the several Underwriters, on the other hand, and does not constitute a recommendation, investment advice, or solicitation of any action by the Underwriters, (b) in connection with the offering of the Securities and the process leading thereto, each Underwriter is and has been acting solely as a principal and is not the agent or fiduciary of the Company, its subsidiaries or their respective stockholders, creditors, employees or any other party, (c) no Underwriter has assumed or will assume an advisory or fiduciary responsibility in favor of the Company with respect to the offering of the Securities or the process leading thereto (irrespective of whether such Underwriter has advised or is currently advising the Company or its subsidiaries on other matters) and no Underwriter has any obligation to the Company with respect to the offering of the Securities except the obligations expressly set forth in this Agreement, (d) the Underwriters and their respective affiliates may be engaged in a broad range of transactions that involve interests that differ from those of the Company and (e) the Underwriters have not provided any legal, accounting, regulatory, investment or tax advice with respect to the offering of the Securities and the Company has consulted its own respective legal, accounting, financial, regulatory and tax advisors to the extent it deemed appropriate, and (f) none of the activities of the Underwriters in connection with the transaction contemplated herein constitutes a recommendation, investment advice or solicitation of any action by the Underwriters with respect to any entity or natural person.



SECTION 13. Recognition of the U.S. Special Resolution Regimes.

(a) In the event that any Underwriter that is a Covered Entity becomes subject to a proceeding under a U.S. Special Resolution Regime, the transfer from such Underwriter of this Agreement, and any interest and obligation in or under this Agreement, will be effective to the same extent as the transfer would be effective under the U.S. Special Resolution Regime if this Agreement, and any such interest and obligation, were governed by the laws of the United States or a state of the United States.

(b) In the event that any Underwriter that is a Covered Entity or a BHC Act Affiliate of such Underwriter becomes subject to a proceeding under a U.S. Special Resolution Regime, Default Rights under this Agreement that may be exercised against such Underwriter are permitted to be exercised to no greater extent than such Default Rights could be exercised under the U.S. Special Resolution Regime if this Agreement were governed by the laws of the United States or a state of the United States.

For purposes of this Section 13, a “BHC Act Affiliate” has the meaning assigned to the term “affiliate” in, and shall be interpreted in accordance with, 12 U.S.C. § 1841(k). “Covered Entity” means any of the following: (i) a “covered entity” as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 252.82(b); (ii) a “covered bank” as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 47.3(b); or (iii) a “covered FSI” as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 382.2(b). “Default Right” has the meaning assigned to that term in, and shall be interpreted in accordance with, 12 C.F.R. §§ 252.81, 47.2 or 382.1, as applicable. “U.S. Special Resolution Regime” means each of (i) the Federal Deposit Insurance Act and the regulations promulgated thereunder and (ii) Title II of the Dodd-Frank Wall Street Reform and Consumer Protection Act and the regulations promulgated thereunder.

SECTION 14. Parties. This Agreement shall inure to the benefit of and be binding upon the Underwriters and the Company and their respective successors. Nothing expressed or mentioned in this Agreement is intended or shall be construed to give any person, firm or corporation, other than the Underwriters and the Company and their respective successors and the controlling persons and officers and directors referred to in Sections 6 and 7 and their heirs and legal representatives, any legal or equitable right, remedy or claim under or in respect of this Agreement or any provision herein contained. This Agreement and all conditions and provisions hereof are intended to be for the sole and exclusive benefit of the Underwriters and the Company and their respective successors, and said controlling persons and officers and directors and their heirs and legal representatives, and for the benefit of no other person, firm or corporation. No purchaser of Securities from any Underwriter shall be deemed to be a successor by reason merely of such purchase.

SECTION 15. Trial by Jury. The Company (on its behalf and, to the extent permitted by applicable law, on behalf of its stockholders and affiliates) and each of the Underwriters hereby irrevocably waives, to the fullest extent permitted by applicable law, any and all right to trial by jury in any legal proceeding arising out of or relating to this Agreement or the transactions contemplated hereby.

SECTION 16. GOVERNING LAW. THIS AGREEMENT AND ANY CLAIM, CONTROVERSY OR DISPUTE ARISING UNDER OR RELATED TO THIS AGREEMENT SHALL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH THE LAWS OF, THE STATE OF NEW YORK WITHOUT REGARD TO ITS CHOICE OF LAW PROVISIONS.

SECTION 17. Consent to Jurisdiction; Waiver of Immunity. Any legal suit, action or proceeding arising out of or based upon this Agreement or the transactions contemplated hereby shall be instituted in (i) the federal courts of the United States of America located in the City and County of New York, Borough of Manhattan or (ii) the courts of the State of New York located in the City and County of New York, Borough of Manhattan (collectively, the "Specified Courts"), and each party irrevocably submits to the exclusive jurisdiction (except for proceedings instituted in regard to the enforcement of a judgment of any such court as to which such jurisdiction is non-exclusive) of such courts in any such suit, action or proceeding. Service of any process, summons, notice or document by mail to such party's address set forth above shall be effective service of process for any suit, action or other proceeding brought in any such court. The parties irrevocably and unconditionally waive any objection to the laying of venue of any suit, action or other proceeding in the Specified Courts and irrevocably and unconditionally waive and agree not to plead or claim in any such court that any such suit, action or other proceeding brought in any such court has been brought in an inconvenient forum.

SECTION 18. TIME. TIME SHALL BE OF THE ESSENCE OF THIS AGREEMENT. EXCEPT AS OTHERWISE SET FORTH HEREIN, SPECIFIED TIMES OF DAY REFER TO NEW YORK CITY TIME.

SECTION 19. Counterparts and Electronic Signatures. This Agreement may be executed in any number of counterparts, each of which shall be deemed to be an original, but all such counterparts shall together constitute one and the same Agreement. Electronic signatures complying with the New York Electronic Signatures and Records Act (N.Y. State Tech. §§ 301-309), as amended from time to time, or other applicable law will be deemed original signatures for purposes of this Agreement. Transmission by telecopy, electronic mail or other transmission method of an executed counterpart of this Agreement will constitute due and sufficient delivery of such counterpart.

SECTION 20. Effect of Headings. The Section headings herein are for convenience only and shall not affect the construction hereof.

*[Signature Page Follows]*

If the foregoing is in accordance with your understanding of our agreement, please sign and return to the Company a counterpart hereof, whereupon this instrument, along with all counterparts, will become a binding agreement among the Underwriters and the Company in accordance with its terms.

Very truly yours,

Y-MABS THERAPEUTICS, INC.

By: /s/ Bo Kruse

Title: Chief Financial Officer

*[Signature Page to Underwriting Agreement]*

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CONFIRMED AND ACCEPTED,  
as of the date first above written:

J.P. MORGAN SECURITIES LLC  
MORGAN STANLEY & CO. LLC  
BOFA SECURITIES, INC.

By: J.P. MORGAN SECURITIES LLC

By: /s/ David Ke  
Authorized Signatory

By: MORGAN STANLEY & CO. LLC

By: /s/ Chris Rigoli  
Authorized Signatory

By: BOFA SECURITIES, INC.

By: /s/ Greg Butz  
Authorized Signatory

For themselves and as Representatives of the other Underwriters named in Schedule A hereto.

*[Signature Page to Underwriting Agreement]*

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SCHEDULE A

The public offering price per share for the Securities shall be \$41.00.

The purchase price per share for the Securities to be paid by the several Underwriters shall be \$38.54, being an amount equal to the public offering price set forth above less \$2.46 per share, subject to adjustment in accordance with Section 2(b) for dividends or distributions declared by the Company and payable on the Initial Securities but not payable on the Option Securities.

Name of Underwriter	Number of Initial Securities
J.P. Morgan Securities LLC	756,098
Morgan Stanley & Co. LLC	756,098
BofA Securities, Inc.	707,317
H.C. Wainwright & Co., LLC	109,756
Kempen & Co U.S.A., Inc.	109,756
Total	<u>2,439,025</u>

Sch. A

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SCHEDULE B-1

Pricing Terms

1. The Company is selling 2,439,025 shares of Common Stock.
2. The Company has granted an option to the Underwriters, severally and not jointly, to purchase up to an additional 365,853 shares of Common Stock.
3. The public offering price per share for the Securities shall be \$41.00.

Sch. B-2

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SCHEDULE B-2

Free Writing Prospectuses

None.

Sch. B-2

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SCHEDULE C

Sch. C

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FORM OF LOCK-UP FROM DIRECTORS, OFFICERS OR OTHER STOCKHOLDERS  
TO BE DELIVERED PURSUANT TO SECTION 5(k)

\_\_\_\_\_, 2021

J.P. Morgan Securities LLC

Morgan Stanley &amp; Co. LLC

BofA Securities, Inc.

as Representatives of the several  
Underwriters to be named in the  
within-mentioned Underwriting Agreement

c/o J.P. Morgan Securities LLC  
383 Madison Avenue  
New York, New York 10179

c/o Morgan Stanley & Co. LLC  
1585 Broadway  
New York, New York 10036

c/o BofA Securities, Inc.  
One Bryant Park  
New York, New York 10036

Re: Proposed Public Offering by Y-mAbs Therapeutics, Inc.

Dear Sirs:

The undersigned, a stockholder, an officer and/or a director, as applicable, of Y-mAbs Therapeutics, Inc., a Delaware corporation (the "Company"), understands that J.P. Morgan Securities LLC ("J.P. Morgan"), Morgan Stanley & Co. LLC ("Morgan Stanley") and BofA Securities, Inc. ("BofA") propose to enter into an Underwriting Agreement (the "Underwriting Agreement") with the Company and the other underwriters party thereto providing for the public offering (the "Public Offering") of shares of the Company's common stock, par value \$0.0001 per share (the "Common Stock"). In recognition of the benefit that the Public Offering will confer upon the undersigned as a stockholder, an officer and/or a director, as applicable, of the Company, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, pursuant to the terms of this letter agreement (the "Lock-Up Agreement"), the undersigned hereby agrees with each underwriter to be named in the Underwriting Agreement (the "Underwriters") that, during the period beginning on the date hereof and ending on the date that is 90 days from the date of the Underwriting Agreement (the "Lock-Up Period"), the undersigned will not, without the prior written consent of J.P. Morgan, Morgan Stanley and BofA, (i) directly or indirectly, offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise transfer or dispose of any shares of the Common Stock or any securities convertible into or exercisable or exchangeable for shares of the Common Stock, whether now owned or hereafter acquired by the undersigned or with respect to which the undersigned has or hereafter acquires the power of disposition (collectively, the "Lock-Up Securities"), or exercise any right with respect to the registration of any of the Lock-up Securities, or file, or cause to be filed any registration statement in connection therewith, under the Securities Act of 1933, as amended (the "Securities Act"), or (ii) enter into any swap or any other agreement or any transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of the Lock-Up Securities, whether any such swap or transaction is to be settled by delivery of shares of the Common Stock or other securities, in cash or otherwise or (iii) publicly disclose the intention to do any of the foregoing described in clauses (i) or (ii).

Notwithstanding the foregoing, and subject to the conditions below, the undersigned may transfer the Lock-Up Securities without the prior written consent of J.P. Morgan, Morgan Stanley and BofA:

- (i) as a bona fide gift or gifts; or
- (ii) to any trust for the direct or indirect benefit of the undersigned or the immediate family of the undersigned or any of their successors upon death, or any partnerships or limited liability company, the partners or members of which consist of or are for the direct or indirect benefit of the undersigned and/or immediate family members or other dependents of the undersigned (for purposes of this Lock-Up Agreement, "immediate family" shall mean any relationship by blood, marriage or adoption, not more remote than first cousin); or
- (iii) as a distribution to partners, members, stockholders or other equity holders of the undersigned; or
- (iv) to another corporation, partnership, limited liability company or other business entity if the undersigned is a corporation, partnership, limited liability company or other business entity, or to the undersigned's affiliates (as such term is defined in Rule 405 as promulgated by the Securities and Exchange Commission (the "SEC") under the Securities Act) or to any investment fund or other entity controlled or managed by the undersigned; or

- (v) if such Lock-Up Securities were acquired by the undersigned in the Public Offering from the Underwriters or on the open market following the effective date of the registration statement for the Public Offering; or
- (vi) pursuant to the exercise, including by “net” exercise, of any options, warrants or other rights to acquire shares of Common Stock, the settlement of any share-settled share appreciation rights or restricted share units or the conversion of any convertible security into Common Stock described in the final prospectus related to the Public Offering (the “Prospectus”), or issued pursuant to an equity plan described in the Prospectus, it being understood that any shares of Common Stock received by the undersigned upon such exercise, settlement or conversion shall be subject to the restrictions on transfers set forth in this Lock-Up Agreement; provided that any filing required under the Securities Exchange Act of 1934, as amended (the “Exchange Act”) shall clearly indicate in the codes and footnotes thereto that (A) the filing relates to the circumstances described above and (B) any disposition of shares in connection with the “net” exercise was made solely to the Company; or
- (vii) pursuant to the sale or transfer to the Company of such number of shares of Common Stock acquired by the undersigned in connection with the exercise of such options or warrants on a “net” exercise basis described in the foregoing clause (vi); provided that any filing required under the Exchange Act shall clearly indicate in the codes and footnotes thereto that (A) the filing relates to the circumstances described above and (B) such sale or transfer, as applicable, in connection with the “net” exercise was made solely to the Company; or
- (viii) pursuant to the sale or transfer to the Company of such number of shares of Common Stock necessary to generate only such amount of cash needed for the payment of taxes (including estimated taxes) due as a result of the exercise or vesting of such options or warrants described in the foregoing clause (vii); provided that any filing required under the Exchange Act shall clearly indicate in the codes and footnotes thereto that (A) the filing relates to the circumstances described above and (B) any disposition of shares in connection with such sale or transfer, as applicable, was made solely to the Company; or
- (ix) pursuant to transfers by operation of law, including pursuant to a qualified domestic relations order, or pursuant to a negotiated divorce settlement; or
- (x) if the undersigned is a trust, pursuant to transfers to a trustor or beneficiary of the trust;
- (xi) pursuant to transfers or dispositions of shares of Common Stock or such other securities by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the undersigned;

- (xii) pursuant to any transactions effectuated pursuant to any contract, instruction or plan that satisfies the requirements of Rule 10b5-1 under the Exchange Act for the transfer of Lock-Up Securities (a "Rule 10b5-1 Plan") established prior to the date of this Lock-Up Agreement, provided that if a filing under Section 16 of the Exchange Act is required by, or voluntarily made with respect to, such transaction, the undersigned shall disclose in such filing that the transaction was effectuated pursuant to a Rule 10b5-1 Plan in existence at the time this Lock-up Agreement was entered into; or
- (xiii) [pursuant to the pledge of up to 587,000 shares of Common Stock as collateral or security pursuant to that certain line of credit maintained by the undersigned with Bank of America, N.A., and any transfer upon foreclosure upon such shares;]<sup>1</sup>

provided that in the case of any transfer or distribution pursuant to clauses (i), (ii), (iii), (iv), (ix), (x) or (xi), each donee, trustee, transferee or distributee shall execute and deliver to J.P. Morgan, Morgan Stanley and BofA a lock-up letter in the form of this Lock-Up Agreement for the balance of the Lock-Up Period; provided, further, that in the case of any transfer or distribution pursuant to clauses (i), (ii), (iii), (iv), (v), (ix), (x) or (xi), no filing by any party (donor, donee, transferor or transferee) under the Exchange Act or other public announcement reporting a reduction in the beneficial ownership shall be required or shall be made voluntarily in connection with such transfer or distribution (other than a filing on a Form 5, Schedule 13G (or 13G/A) or 13F made after the expiration of the Lock-Up Period); and provided, further, that any transfer or distribution pursuant to clauses (i), (ii), (iii), (iv), (x) or (xi) shall not involve a disposition for value.

In addition, this Lock-Up Agreement shall not prohibit the undersigned from establishing, during the Lock-Up Period, a Rule 10b5-1 Plan; provided that (1) no sales of Lock-Up Securities or other transactions shall be made pursuant to a Rule 10b5-1 Plan prior to the expiration of the Lock-Up Period, and (2)(A) the Company is not required to, and does not voluntarily, report the establishment of such Rule 10b5-1 Plan with the SEC during the Lock-Up Period and (B) no filing shall be voluntarily made by or on behalf of the undersigned during the Lock-Up Period.

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<sup>1</sup>To be included in Lock-Up Agreement executed by one of the Company's officers.

[TO BE INCLUDED IN LOCK-UP EXECUTED BY MAJOR HOLDERS (AS DEFINED BELOW): The restrictions contained herein shall not apply to any transfers, sales, tenders or other dispositions of Common Stock or any security convertible into or exercisable or exchangeable for Common Stock pursuant to a bona fide third-party tender offer, merger, amalgamation, consolidation or other similar transaction made to or involving all holders of the Common Stock or such other securities pursuant to a change of control of the ownership of the Company (including, without limitation, the entry into any lock-up, voting or similar agreement pursuant to which the undersigned may agree to transfer, sell, tender or otherwise dispose of Common Stock or other such securities in connection with any such transaction, or vote any securities in favor of any such transaction); provided that if such tender offer, merger, amalgamation, consolidation or other similar transaction is not completed, any Common Stock or any security convertible into or exercisable or exchangeable for Common Stock subject to this Lock-Up Agreement shall remain subject to the restrictions contained in this Lock-Up Agreement. For purposes of this Lock-Up Agreement, “change of control” shall mean the consummation of any bona fide third-party tender offer, merger, amalgamation, consolidation or other similar transaction, in one transaction or a series of related transactions, the result of which is that any “person” (as defined in Section 13(d)(3) of the Exchange Act), or group of affiliated persons, other than the Company or an Underwriter in the Public Offering, becomes the beneficial owner (as defined in Rules 13d-3 and 13d-5 of the Exchange Act) of greater than 50% of the total voting power of the outstanding voting securities of the Company or the surviving entity.]

This Lock-Up Agreement shall automatically terminate upon the earliest to occur, if any, of (1) either the Company, on the one hand, or J.P. Morgan, Morgan Stanley and BofA on behalf of the Underwriters, on the other hand, advising the other in writing, prior to the execution of the Underwriting Agreement, that they have determined not to proceed with the Public Offering, (2) termination of the Underwriting Agreement (other than the provisions thereof which survive termination) before the payment for and delivery of the Common Stock to be sold thereunder, (3) the withdrawal of the registration statement filed with the SEC with respect to the Public Offering, or (4) April 30, 2021, in the event that the Underwriting Agreement has not been executed by that date.

The undersigned further acknowledges and agrees that, although the Representatives may be required or choose to provide certain Regulation Best Interest and Form CRS disclosures to you in connection with the Public Offering, the Representatives and the other Underwriters are not making a recommendation to you to enter into this Lock-Up Agreement, participate in the Public Offering, or sell any shares at the price determined in the Public Offering, and nothing set forth in such disclosures is intended to suggest that the Representatives or any Underwriter is making such a recommendation.

The undersigned also agrees and consents to the entry of stop transfer instructions with the Company’s transfer agent and registrar against the transfer of the Lock-Up Securities except in compliance with the foregoing restrictions.

This Lock-Up Agreement and any claim, controversy or dispute arising under or related to this Lock-Up Agreement shall be governed by and construed in accordance with the laws of the State of New York, without regard to the conflict of laws principles thereof.

If any record or beneficial owner of any securities of the Company is granted an early release from the restrictions described herein during the Lock-Up Period with respect to an aggregate amount of securities of the Company in excess of one percent of the issued and outstanding shares of the Company (whether in one or multiple releases), then each Major Holder (as defined below) shall also be granted an early release from its obligations hereunder on a pro rata basis with all other record or beneficial holders of similarly restricted securities of the Company based on the maximum percentage of shares held by any such record or beneficial holder being released from such holder's lock-up agreement; provided, however, that in the case of an early release from the restrictions described herein during the Lock-Up Period in connection with an underwritten public offering, whether or not such offering or sale is wholly or partially a secondary offering of the Company's Common Stock (an "Underwritten Sale"), such early release shall only apply with respect to such Major Holder's participation in such Underwritten Sale. Notwithstanding any other provisions of this lock-up agreement, if J.P. Morgan, Morgan Stanley and BofA determine that a record or beneficial owner of any securities should be granted an early release from a lock-up agreement due to circumstances of an emergency or hardship, then the Major Holders shall not have any right to be granted an early release pursuant to the terms of this paragraph. For purposes of this lock-up agreement, each of the following persons is a "Major Holder": each record or beneficial owner, as of the date hereof, of more than 5% of the outstanding shares of securities of the Company (for purposes of determining record or beneficial ownership of a stockholder, all shares of securities held by investment funds affiliated with such stockholder shall be aggregated).

The undersigned hereby consents to receipt of this Lock-Up Agreement in electronic form and understands and agrees that this Lock-Up Agreement may be signed electronically. In the event that any signature is delivered by facsimile transmission, electronic mail, or otherwise by electronic transmission evidencing an intent to sign this Lock-Up Agreement, such facsimile transmission, electronic mail or other electronic transmission shall create a valid and binding obligation of the undersigned with the same force and effect as if such signature were an original. Execution and delivery of this Lock-Up Agreement by facsimile transmission, electronic mail or other electronic transmission is legal, valid and binding for all purposes.

*[Signature Page Follows]*

Very truly yours,  
[NAME OF STOCKHOLDER/OFFICER/DIRECTOR]

By: \_\_\_\_\_  
Name:  
Title:

If not signing in an individual capacity:

\_\_\_\_\_  
Name of Authorized Signatory (Print)

\_\_\_\_\_  
Title of Authorized Signatory (Print)  
*(Indicate capacity of person signing if signing as custodian, trustee, or on behalf of an entity.)*

NEW YORK  
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SINGAPORE  
PHILADELPHIA  
CHICAGO  
WASHINGTON, DC  
SAN FRANCISCO  
SILICON VALLEY  
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MYANMAR

ALLIANCES IN MEXICO  
AND SRI LANKA

Y-mAbs Therapeutics, Inc.  
230 Park Avenue  
Suite 3350  
New York, NY 10169

Ladies and Gentlemen:

We have acted as counsel to Y-mAbs Therapeutics, Inc., a Delaware corporation (the “Company”), with respect to certain matters in connection with the offering by the Company of up to 2,439,025 shares of the Company’s common stock, par value \$0.0001 (the “Shares”), including up to 365,853 Shares that may be sold pursuant to the exercise of an option to purchase additional Shares pursuant to a Registration Statement on Form S-3 (Registration Statement No. 333-234034), as amended (the “Registration Statement”), filed with the Securities and Exchange Commission (the “Commission”) under the Securities Act of 1933, as amended (the “Act”), the prospectus included in the Registration Statement (the “Base Prospectus”), and the prospectus supplement relating to the Shares dated February 17, 2021 filed with the Commission pursuant to Rule 424(b) under the Act (the “Prospectus Supplement” and together with the Base Prospectus, the “Prospectus”). All of the Shares are to be sold pursuant to the proposed form of Underwriting Agreement by and among the Company and J.P. Morgan Securities LLC, Morgan Stanley & Co. LLC and BofA Securities, Inc., as representatives of the several underwriters listed on Schedule A thereto (the “Underwriting Agreement”) filed as Exhibit 1.1 to the Registration Statement.

For purposes of rendering this opinion, we have examined originals or copies (certified or otherwise identified to our satisfaction) of: (a) the Underwriting Agreement; (b) the Amended and Restated Certificate of Incorporation of the Company, as filed with and certified by the Secretary of State of the State of Delaware; (c) the Amended and Restated Bylaws of the Company in the form filed with the Commission; and (d) resolutions of the Board of Directors of the Company, as attested by the Secretary of the Company.

DUANE MORRIS LLP

230 PARK AVENUE, SUITE 1130 NEW YORK, NY 10169-0079

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We have also examined originals or copies, certified or otherwise identified to our satisfaction, of such documents, corporate records, certificates of public officials and other instruments as we have deemed necessary for the purposes of rendering this opinion. In our examination, we have assumed the genuineness of all signatures, the authenticity of all documents submitted to us as originals and the conformity with the originals of all documents submitted to us as copies.

As to questions of fact material to this opinion, we have relied only upon the above referenced certificate and documents and upon the representations and warranties of the Company included in the Underwriting Agreement.

The opinion expressed herein is limited in all respects to the Delaware General Corporation Law, as amended, including all reported judicial decisions interpreting such law. We express no opinion herein as to the effect on the matters covered by this letter of any other laws of the State of Delaware or the laws of any other jurisdiction, domestic or foreign.

Based upon the foregoing, and subject to the qualifications stated herein, we are of the opinion that:

Following (i) execution and delivery of the Underwriting Agreement by all parties thereto, (ii) effectiveness of the Registration Statement, (iii) issuance of the Shares pursuant to the terms of the Underwriting Agreement, and (iv) receipt by the Company of the consideration for the Shares specified in the resolutions of the Board of Directors of the Company and the pricing committee thereof, the Shares when sold and issued in accordance with the Underwriting Agreement, Registration Statement and the Prospectus, will be validly issued, fully paid and nonassessable.

The opinion expressed herein is rendered as of the date hereof and is based on existing law, which is subject to change. Where our opinion expressed herein refers to events to occur at a future date, we have assumed that there will have been no changes in the relevant law or facts between the date hereof and such future date. We do not undertake to advise you of any changes in the opinion expressed herein from matters that may hereafter arise or be brought to our attention or to revise or supplement such opinion should the present laws of any jurisdiction be changed by legislative action, judicial decision or otherwise.

Our opinion expressed herein is limited to the matters expressly stated herein, and no opinion is implied or may be inferred beyond the matters expressly stated.

This opinion is for your benefit in connection with the Registration Statement and may be relied upon only by you and by persons entitled to rely upon it pursuant to the applicable provisions of the Act.

We consent to the filing of this opinion as Exhibit 5.1 to the Registration Statement, and we consent to the reference of our name under the caption "Legal Matters" in the Prospectus forming a part of the Registration Statement. In giving this consent, we do not admit that we are "experts" within the meaning of Section 11 of the Securities Act or within the category of persons whose consent is required under Section 7 of the Securities Act.

Very truly yours,

/s/ Duane Morris LLP

**ASSET PURCHASE AGREEMENT**  
**BY AND BETWEEN**  
**UNITED THERAPEUTICS CORPORATION**

**AND**

**Y-MABS THERAPEUTICS, INC.**

**December 24, 2020**

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List of Exhibits

Exhibit 2.5(a)	Form of Seller FDA Transfer Notification
Exhibit 2.5(b)	Form of Seller Closing Certificate
Exhibit 2.6(b)	Form of Buyer FDA Transfer Notification
Exhibit 2.6(c)	Form of Buyer Closing Certificate
Exhibit 5.12	Form of Pending Transfer Notification

## ASSET PURCHASE AGREEMENT

This ASSET PURCHASE AGREEMENT (this “*Agreement*”) is made and entered into as of December 24, 2020 (“*Effective Date*”), by and between **United Therapeutics Corporation**, a Delaware corporation (“*Buyer*”), and **Y-mAbs Therapeutics, Inc.**, a Delaware corporation (“*Seller*”). Buyer and Seller may hereinafter be referred to individually as a “*Party*” and collectively as the “*Parties*”.

### RECITALS

**WHEREAS**, Seller is the sole and exclusive owner of a Priority Review Voucher (as defined below).

**WHEREAS**, Seller and Buyer each (i) desire that Buyer purchase from Seller, and Seller sell, transfer and assign to Buyer, the Priority Review Voucher and all rights, benefits and entitlements appurtenant thereto, all on the terms set forth herein (such transaction, the “*Asset Purchase*”) and (ii) in furtherance thereof, have adopted and approved this Agreement and, upon the terms and subject to the conditions set forth in this Agreement, have approved the Asset Purchase as contemplated by this Agreement in accordance with all applicable Legal Requirements.

**WHEREAS**, Seller and Buyer desire to make certain representations, warranties, covenants and other agreements as set forth herein in connection with the Asset Purchase contemplated by this Agreement.

**NOW, THEREFORE**, in consideration of the foregoing and their mutual undertakings hereinafter set forth, and intending to be legally bound, the Parties hereto agree as follows:

### ARTICLE 1 DEFINITIONS

**1.1** Certain Definitions. As used in this Agreement, the following capitalized terms shall have the meanings indicated below:

(a) “*Action*” means any claim, audit, examination, action, cause of action or suit (whether in contract or tort or otherwise), litigation (whether at law or in equity, whether civil or criminal), assessment, arbitration, mediation, investigation, hearing, charge, complaint, demand, notice or proceeding.

(b) “*Affiliate*” means any Person which, directly or indirectly through one or more intermediaries, controls, is controlled by, or is under common control with, a Party to this Agreement, for so long as such control exists, whether such Person is or becomes an Affiliate on or after the Effective Date. A Person shall be deemed to “control” another Person if it: (i) owns, directly or indirectly, beneficially or legally, at least fifty percent (50%) of the outstanding capital stock, voting securities or other ownership interest (or such lesser percentage which is the maximum allowed to be owned by such Person in a particular jurisdiction) of such other Person (or, with respect to a limited partnership or other similar entity, its general partner or controlling entity); or (ii) has the power, whether pursuant to Contract, ownership of securities or otherwise, to direct the management and policies of such other Person.

(c) “**Alternative Transaction**” means, other than the transactions contemplated by this Agreement, any proposal or offer from any Person or group of Persons (other than Buyer or its Affiliates or their respective representatives) for any acquisition by, or transfer, license or other grant of rights to, such Person or group of Persons of any right, title or interest in or to the Purchased Assets; *provided* that “**Alternative Transaction**” shall not include any acquisition of Seller (whether through a stock purchase, merger, sale of all or substantially all assets or otherwise).

(d) “**Antitrust Approval**” has the meaning set forth in Section 5.5(b).

(e) “**Business Day**” means a day (i) other than Saturday or Sunday and (ii) on which commercial banks are open for business in New York, New York.

(f) “**Buyer Indemnified Party**” has the meaning set forth in Section 8.1(a).

(g) “**Closing**” has the meaning set forth in Section 2.3.

(h) “**Closing Date**” has the meaning set forth in Section 2.3.

(i) “**Confidential Information**” means (i) any and all confidential and proprietary information, including data, results, conclusions, know-how, experience, financial information, plans and forecasts, that may be delivered, made or communicated by or on behalf of a Party related to the subject matter hereof or otherwise in connection with this Agreement and (ii) the terms, conditions and existence of this Agreement.

(j) “**Consent**” means any and all filings, authorizations, consents, approvals, notices, permits, orders, registrations or declarations.

(k) “**Contract**” means any written or oral legally binding contract, agreement, instrument, commitment or undertaking (including leases, licenses, mortgages, notes, guarantees, sublicenses, subcontracts and purchase orders).

(l) “**Effective Date**” has the meaning set forth in the Preamble.

(m) “**Encumbrance**” means any lien, pledge, charge, mortgage, easement, encroachment, imperfection of title, title exception, title defect, right of possession, lease, security interest, encumbrance, adverse claim, interference or restriction on use arising out of any Contract or Legal Requirement, or restriction on transfer.

(n) “**FDA**” means the United States Food and Drug Administration.

(o) “**FFDCA**” means the United States Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 *et seq.*, as amended from time to time, together with any rules, regulations and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

(p) “**Governmental Entity**” means any supranational, national, state, municipal, local or foreign government, any court, tribunal, arbitrator, administrative agency, commission or other governmental official, authority or instrumentality, in each case whether domestic or foreign, any stock exchange or similar self-regulatory organization or any quasi-governmental, private body or arbitral body exercising any executive, legislative, judicial, quasi-judicial, regulatory, taxing, importing, administrative or other governmental or quasi-governmental authority.



(q) “**HSR Act**” means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended from time to time.

(r) “**Indemnified Party**” means any of the Buyer Indemnified Parties or Seller Indemnified Parties, as applicable.

(s) “**Indemnifying Party**” means any Person against whom a claim for indemnification is being asserted under any provision of ARTICLE 8.

(t) “**Judgment**” means any orders, writs, injunctions, awards, judgments, stipulations, determinations and decrees entered by or with any Governmental Entity.

(u) “**Knowledge**” means, with respect to Seller, the actual knowledge of the facts and information of any director or officer of Seller, after performing a reasonable inquiry with respect to such facts and information.

(v) “**Law**” means any federal, state, foreign, local, municipal or other law, statute, constitution, principle of common law, resolution, ordinance, code, edict, decree, rule, regulation, ruling or requirement issued, enacted, adopted, promulgated, implemented or otherwise put into effect by or under the authority of any Governmental Entity.

(w) “**Legal Requirements**” means any Law, or any Judgment, or any license, franchise, authorization of any Governmental Entity or similar right granted under any of the forgoing, or any similar provision having the force or effect of law. Legal Requirements shall include, with respect to Seller, any requirements relating to the Priority Review Voucher set forth in the FFDCA or the letter from the Department of Health and Human Services to Seller, BLA 761171 FDA Approval Letter, for DANYELZA<sup>®</sup> (naxitamab-gqgk).

(x) “**Liabilities**” means all debts, liabilities and obligations, whether presently in existence or arising hereafter, accrued or fixed, absolute or contingent, matured or unmatured, determined or determinable, asserted or unasserted, known or unknown, including those arising under any law, action or governmental order and those arising under any Contract.

(y) “**Losses**” means all losses, Liabilities, damages, claims, causes of action, judgments, awards, suits, Taxes, fines, penalties, costs or expenses (including reasonable attorneys’ and experts’ fees and expenses).

(z) “**Party**” and “**Parties**” have the meanings set forth in the Preamble.

(aa) “**Pending Transfer Notification**” has the meaning set forth in Section 5.12.

(bb) “**Person**” means any natural person, company, corporation, limited liability company, general partnership, limited partnership, trust, proprietorship, joint venture, business organization or Governmental Entity.

(cc) “**Pre-Closing Period**” has the meaning set forth in Section 5.1.

(dd) “**Priority Review**” means a priority review of and action upon a human drug application by the FDA not later than six (6) months after the 60 day filing date of such application to the FDA, as defined in the FFDCA.

(ee) “**Priority Review Voucher**” means the rare pediatric disease priority review voucher issued by the United States Secretary of Health and Human Services to Seller, as evidenced in the Federal Register, Vol. 85, No. 235, page 78859, tracking number PRV BLA 761171, as the sponsor of a rare pediatric disease product application, that entitles the holder of such voucher to Priority Review of a single human drug application submitted under section 505(b)(1) or section 351(a) of the United States Public Health Service Act, as further defined in the FFDCA.

(ff) “**Proceeding**” means any action, arbitration, audit, hearing, investigation, litigation or suit (whether civil, criminal, administrative, judicial or investigative, whether formal or informal, whether public or private) commenced, brought, conducted or heard by or before, or otherwise involving, any Governmental Entity or arbitrator.

(gg) “**Purchased Assets**” means certain intangible assets, consisting of the Priority Review Voucher, including any and all rights, benefits and entitlements afforded to the holder thereof.

(hh) “**Purchase Price**” has the meaning set forth in Section 2.2.

(ii) “**Seller Indemnified Party**” has the meaning set forth in Section 8.1(b).

(jj) “**Tax**” or “**Taxes**” means any net income, alternative or add-on minimum tax, gross income, gross receipts, sales, use, value added tax, ad valorem, transfer, franchise, profits, license, withholding, payroll, employment, excise, severance, stamp, occupation, municipal tax, municipal surcharge premium, property, environmental or windfall profit tax, social security contribution or other tax of any kind whatsoever, together with any interest or any penalty, addition to tax or additional amount in the nature of a tax imposed by any Governmental Entity responsible for the imposition of any such tax (domestic or foreign), whether disputed or not and including (i) the Tax Liability of any other Person imposed pursuant to Treasury Regulations Section 1.1502-6 or any similar provision of other Tax Law, and (ii) the obligation to indemnify or assume or otherwise succeed to the Tax Liability of any other Person, by contract or pursuant to any Law.

(kk) “**Third Party Claim**” has the meaning set forth in Section 8.2(b).

Other capitalized terms defined elsewhere in this Agreement and not defined in this Section 1.1 shall have the meanings assigned to such terms in this Agreement.

## ARTICLE 2 PURCHASE AND SALE; CLOSING

### 2.1 Purchase and Sale of Purchased Assets.

(a) Upon the terms and subject to the conditions of this Agreement, at and as of the Closing, Buyer shall purchase from Seller and Seller shall sell, transfer, convey, assign and deliver to Buyer at the Closing all of the Purchased Assets free and clear of all Encumbrances. Seller shall perform all actions necessary to facilitate the transfer of the Purchased Assets to Buyer.

(b) Buyer shall not assume, nor shall it be liable for, or otherwise be obligated to pay, perform or discharge, any Liabilities of Seller or its Affiliates, including any Liabilities arising from or related to Seller’s ownership prior to the Closing of any rights with respect to the Purchased Assets. Seller shall be solely responsible for all such Liabilities.

**2.2 Purchase Price.** The total consideration to be paid by Buyer for all of the Purchased Assets shall be ONE HUNDRED FIVE MILLION U.S. DOLLARS (U.S. \$105,000,000) (the “**Purchase Price**”). Buyer shall pay the Purchase Price to Seller on the Closing Date in cash by wire transfer of immediately available funds to a bank account of Seller in accordance with the wire instructions provided by the Seller to the Buyer on or before the date hereof.

**2.3 Closing.** The closing of the transactions contemplated hereby (the “**Closing**”) shall take place remotely via the exchange of documents and signatures, at 10:00 a.m. eastern time on the third (3<sup>rd</sup>) Business Day following the date on which all of the conditions precedent set forth in ARTICLE 6 have been satisfied or waived (other than conditions to be satisfied only by the delivery of certificates or other documents at the Closing, but subject to the satisfaction or waiver of such conditions at the Closing), or at such other time and place as Buyer and Seller may agree upon in writing (the “**Closing Date**”).

**2.4 Title Passage; Delivery of Purchased Assets.**

(a) Title Passage. Upon the Closing, all of the right, title and interest of Seller in and to the Purchased Assets shall pass to Buyer.

(b) Method of Delivery of Assets. Within one (1) Business Day of the Closing Date, Seller and Buyer will each submit to the FDA the separate notifications referred to in Section 2.5(a) and Section 2.6(b), respectively.

(c) Filings; Notifications. Buyer and Seller agree to cooperate and assist each other with respect to all filings or notifications to FDA related to the transfer and assignment of the Purchased Assets.

**2.5 Closing Deliveries by Seller.** At the Closing, Seller shall deliver to Buyer the following:

(a) a copy of the notification of the purchase and sale of the Priority Review Voucher pursuant to this Agreement to be submitted to the FDA by Seller pursuant to Section 2.4(b), which notification shall be in the form of Exhibit 2.5(a) or such other form as the FDA may require as of the Closing Date;

(b) a certificate of Seller dated as of the Closing Date, in the form set out in Exhibit 2.5(b), duly executed by Seller, certifying as to the satisfaction of the conditions set forth in Sections 6.2(a) and 6.2(b); and

(c) such other documents and instruments as may be required to be delivered by Seller by any other provision of this Agreement or as may be reasonably required to consummate the transactions contemplated by this Agreement.

**2.6 Closing Deliveries by Buyer.** At the Closing, Buyer shall deliver to Seller the following:

(a) payment of the Purchase Price in accordance with Section 2.2;

(b) a copy of the notification of the purchase and sale of the Priority Review Voucher pursuant to this Agreement to be submitted to the FDA by Buyer pursuant to Section 2.4(b), which notification shall be in the form of Exhibit 2.6(b) or such other form as the FDA may require as of the Closing Date;

(c) a certificate of Buyer dated as of the Closing Date, in the form set out in Exhibit 2.6(c), duly executed by Buyer, certifying as to the satisfaction of the conditions set forth in Sections 6.1(a) and 6.1(b); and

(d) such other documents and instruments as may be required to be delivered by Buyer by any other provision of this Agreement or as may be reasonably required to consummate the transactions contemplated by this Agreement.

**2.7** Withholding. Notwithstanding anything to the contrary in this Agreement, Buyer and its agents shall be entitled to deduct and withhold from any amounts payable in connection with the sale of the Purchased Assets to Buyer that are required to be deducted and withheld with respect to the making of payments pursuant to this Agreement as required by any provision of federal, state, local or foreign Law relating to Taxes. To the extent amounts are so withheld, the withheld amounts shall be treated for all purposes of this Agreement as having been paid to Seller. If Buyer determines that it intends to deduct or withhold any amount from the Purchase Price, Buyer will provide written notice to Seller prior to payment. Prior to making any deduction or withholding from any payment to Seller, Buyer shall use commercially reasonable efforts to provide five (5) Business Days' prior written notice to Seller setting forth the amounts subject to deduction or withholding, a brief explanation of the applicable Law and to provide to Seller a reasonable opportunity within such five (5) Business Days to provide forms or other evidence that would exempt such amounts from such deduction or withholding under applicable Law.

**2.8** Transfer Taxes. Notwithstanding anything to the contrary in this Agreement, all transfer, documentary, sales, use, stamp, value added, goods and services, excise, registration and other similar taxes, and all conveyance fees, recording charges and other fees and charges (including penalties and interest) incurred in connection with the sale of the Purchased Assets ("**Transfer Taxes**") shall be borne fifty percent (50%) by Seller and fifty (50%) by Buyer. The Parties hereto agree to reasonably cooperate to minimize any Transfer Taxes to the extent permitted by applicable Law.

### **ARTICLE 3 REPRESENTATIONS AND WARRANTIES OF SELLER**

Seller represents and warrants to Buyer, as of the Effective Date and as of the Closing Date (except, in each case, to the extent such representations and warranties speak expressly as of a different date, and then, as of such date), as follows:

**3.1** Organization, Standing and Power. Seller is a corporation duly organized and validly existing under the laws of Delaware. Seller has the corporate power and authority to own, operate and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect any of the Purchased Assets or Buyer's ownership and rights with respect to, or use of, any of the Purchased Assets after the Closing. Seller is not in violation of its certificate of incorporation or bylaws, in each case as amended to date.

**3.2** Due Authority. Seller has all requisite corporate power and authority to execute and deliver, perform its obligations under, and consummate the transactions contemplated by, this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase, have been duly and validly approved and authorized by the Board of Directors of Seller, and no other actions or proceedings on the part of Seller, the Board of Directors of Seller or any of the stockholders of Seller are necessary to approve or authorize the execution, delivery and performance of this Agreement, or the consummation of the transactions contemplated hereunder. This Agreement has been duly executed and delivered by Seller. This Agreement, upon execution by the Parties, will constitute a valid and binding obligation of Seller enforceable against Seller in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar laws affecting the rights of creditors generally and (b) rules of law governing specific performance, injunctive relief and other equitable remedies.

**3.3** No Contravention. The execution and delivery by Seller of this Agreement does not, and the consummation of the transactions contemplated hereby, including the transfer of title to, ownership in, and possession of the Purchased Assets, will not, (a) result in the creation of any Encumbrance on any of the Purchased Assets or (b) conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, cancellation or acceleration of any obligation or loss of any benefit under, or require any consent, approval or waiver from any Person pursuant to, (i) any provision of the certificate of incorporation or bylaws or other organizational or governing documents of Seller, in each case as amended to date, (ii) any Contract to which Seller is a party or bound by or by which its assets or properties are bound or under which Seller has material rights or benefits or (iii) any Legal Requirements applicable to Seller or any of its assets or properties.

**3.4** No Consents. Except for (a) any Antitrust Approvals required by the HSR Act or any other applicable Legal Requirements relating to competition, merger control or antitrust, and (b) the letters referenced in Section 2.4(b), no Consent of a Governmental Entity or any other Person, on the part of Seller is necessary or required in connection with the execution, delivery and performance by Seller of this Agreement, and the consummation by Seller of the transactions contemplated hereby.

**3.5** Title to Purchased Assets. Seller is the sole and exclusive owner of the Purchased Assets and owns good and transferable title to the Purchased Assets free and clear of any Encumbrances. Seller has performed all actions necessary to perfect its ownership of, and its ability to transfer, the Purchased Assets. Seller has the full right to sell, transfer, convey, assign and deliver the Purchased Assets to Buyer at the Closing, and Seller will sell, transfer, convey, assign and deliver the Purchased Assets to Buyer at the Closing, in each case, free and clear of all Encumbrances.

**3.6** Contracts. Except for this Agreement, there is no Contract to which Seller or any of its Affiliates is a party to or bound by that involves or affects (or may involve or affect) the issuance of, ownership of, transfer of, title to, or use of any of the Purchased Assets, or that otherwise assigned, transferred, licensed, conveyed or encumbered, or granted or allowed to exist any Encumbrance with respect to, any of its right, title or interest in, to or under the Purchased Assets.

**3.7** Compliance With Legal Requirements. Seller and its Affiliates are, and at all times have been, in compliance with all Legal Requirements that are or were applicable to the Purchased Assets or such Person's conduct, acts, or omissions with respect to the Purchased Assets. None of Seller or any of its Affiliates has received any written or, to Seller's Knowledge, oral notice or other communication from any Person regarding any actual, alleged, possible or potential violation of, or failure to comply with, any such Legal Requirement.

**3.8** Legal Proceedings. There is no pending, or to Seller's Knowledge, threatened, Action involving Seller or any of its Affiliates, nor has there been an Action involving Seller or any of its Affiliates, and neither Seller nor any of its Affiliates are a party or subject to the provisions of any Judgment, (a) that involves or affects (or may involve or affect) the issuance of, ownership of, transfer of, title to, or use of any of the Purchased Assets, or (b) challenging the transactions contemplated by this Agreement. To the Knowledge of Seller, there is no fact or circumstance that would reasonably be expected to serve as a basis for any of the foregoing Actions.

**3.9** Governmental Authorizations. Neither Seller nor any of its Affiliates is required to hold any license, registration, or permit issued by any Governmental Entity to own, use or transfer the Purchased Assets, other than such licenses, registrations or permits that have already been obtained.

**3.10** Solvency. Seller is not entering into this Agreement with the actual intent to hinder, delay, or defraud any creditor of Seller. The remaining assets of Seller after the Closing will not be unreasonably small in relation to the business in which Seller will engage after the Closing. Upon and immediately following the Closing Date, after giving effect to all of the transactions contemplated by and in this Agreement (including the payment of the Purchase Price), Seller will not be insolvent and will have sufficient capital to continue in business and pay its debts as they become due.

**3.11** Revocation; Use of Purchased Assets. The Priority Review Voucher has not been terminated, canceled or revoked and to Seller's Knowledge there are no facts or circumstances that could reasonably be expected to (with or without notice or lapse of time, or both) give rise to a right of FDA to terminate, cancel or revoke the Priority Review Voucher or that could reasonably be expected to preclude (a) the sale of the Purchased Assets to Buyer, or (b) Buyer's use of the Purchased Assets to obtain Priority Review or any other benefit associated with the Purchased Assets. Neither Seller nor any of its Affiliates has notified FDA of its own intent to use the Priority Review Voucher.

**3.12** Marketed Product. Seller has initiated marketing in the United States of the rare pediatric disease product for which the Priority Review Voucher was awarded within the three hundred sixty five (365)-day period beginning on the date of the FDA approval of such rare pediatric disease product and has continuously marketed such product in the United States since Seller initiated marketing of such product. The rare pediatric disease product application for which the Priority Review Voucher was awarded was not submitted by Seller to the FDA prior to the date that is ninety (90) days after the date of enactment of the Prescription Drug User Fee Amendments of 2012.

**3.13** Brokers. Except for Jefferies LLC, the fees and expenses of which shall be paid by Seller, no broker, finder or investment banker is entitled to any brokerage or finder's fee in connection with the purchase and sale of the Purchased Assets hereunder or any of the other transactions contemplated by this Agreement based upon arrangements made by or on behalf of Seller.

**3.14** No Other Representations. Neither Seller nor any of its Representatives is making any representation or warranty of any kind or nature whatsoever, oral or written, express or implied, except as otherwise expressly set forth in this ARTICLE 3, and Seller hereby disclaims any such other representations and warranties.

#### **ARTICLE 4 REPRESENTATIONS AND WARRANTIES OF BUYER**

Buyer represents and warrants to Seller, as of the Effective Date and as of the Closing Date (except, in each case, to the extent such representations and warranties speak expressly as of a different date, and then, as of such date), as follows:

**4.1** Organization, Standing and Power. Buyer is a corporation duly organized, validly existing and in good standing under the laws of Delaware.

**4.2** Authority. Buyer has all requisite corporate power and authority to execute and deliver, perform its obligations under, and consummate the transactions contemplated by, this Agreement. The execution, delivery and performance of, and the consummation of the transactions contemplated by, this Agreement have been duly and validly approved by all necessary corporate action on the part of Buyer, and no other actions or proceedings on the part of Buyer, the Board of Directors of Buyer or its Affiliates, or any of the stockholders of Buyer or its Affiliates are necessary to approve or authorize the execution, delivery and performance of this Agreement, or the consummation of the transactions contemplated hereunder. This Agreement has been duly executed and delivered by Buyer. This Agreement, upon execution by the Parties, will constitute a valid and binding obligation of Buyer, enforceable against Buyer in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar laws affecting the rights of creditors generally and (b) rules of law governing specific performance, injunctive relief and other equitable remedies.

**4.3** No Contravention. The execution and delivery by Buyer of this Agreement does not, and the consummation of the transactions contemplated hereby will not, conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, cancellation or acceleration of any obligation or loss of any benefit under, or require any consent, approval or waiver from any Person pursuant to, (a) any provision of the organizational or governing documents of Buyer, in each case as amended to date, (b) any Contract to which Buyer is a party or bound by or by which its assets or properties are bound or under which Buyer has rights or benefits (except as would not reasonably be expected to have a material adverse effect on the ability of Buyer to consummate the purchase of the Purchased Assets at Closing) or (c) any Legal Requirements applicable to Buyer or any of its assets or properties (except as would not reasonably be expected to have a material adverse effect on the ability of Buyer to consummate the purchase of the Purchased Assets at Closing).

**4.4** No Consents. Except for (a) any Antitrust Approvals required by the HSR Act or any other applicable Legal Requirements relating to competition, merger control or antitrust, (b) the letters referenced in Section 2.4(b), and (c) any Consents the absence of which would not reasonably be expected to have a material adverse effect on the ability of Buyer to consummate the purchase of the Purchased Assets at Closing, no Consent of any Governmental Entity or any other Person is required by or with respect to Buyer in connection with the execution, delivery and performance by Buyer of this Agreement or the consummation by Buyer of the transactions contemplated hereby.

**4.5** Brokers. No broker, finder or investment banker is entitled to any brokerage or finder's fee in connection with the purchase and sale of the Purchased Assets hereunder or any of the other transactions contemplated by this Agreement based upon arrangements made by or on behalf of Buyer.

**4.6** Financing. Buyer has sufficient funds to permit the Buyer to consummate the transactions contemplated by this Agreement. Notwithstanding anything to the contrary contained herein, the Parties acknowledge and agree that it shall not be a condition to the obligations of Buyer to consummate the transactions contemplated hereby that Buyer have sufficient funds for payment of the Purchase Price

**4.7** Non-Reliance. Neither Seller nor any of its Affiliates nor any of their Representatives makes, or has made any representation or warranty, oral or written, express or implied, as to the accuracy or completeness of any information concerning the Purchased Assets contained herein or made available in connection with Buyer's investigation of the foregoing, except as expressly set forth in this Agreement, and Seller, its Affiliates and their Representatives expressly disclaim any and all liability that may be based on such information or errors therein or omissions therefrom. Buyer has not relied and is not relying on any statement, representation or warranty, oral or written, express or implied (including any representation or warranty as to merchantability or fitness for a particular purpose), made by Seller, any of its Affiliates or any of their Representatives, except as expressly set forth in ARTICLE 3. Neither Seller nor its Affiliates nor any of their Representatives shall have or be subject to any liability to Buyer or any other Person resulting from the distribution to Buyer, or Buyer's use of, any information, documents or materials made available to Buyer, whether orally or in writing, in any presentations, due diligence discussions or in any other form in expectation of, or in connection with, the Asset Purchase, other than as expressly set forth in this Agreement.

## **ARTICLE 5 COVENANTS**

**5.1** Negative Covenants of Seller. During the period from the Effective Date and continuing until the earlier of the termination of this Agreement or the Closing Date (the "*Pre-Closing Period*"), except as otherwise expressly contemplated by this Agreement or with Buyer's prior written consent, which consent shall not be unreasonably withheld or delayed, Seller shall not, and shall cause its Affiliates not to, knowingly take or permit any action that, or omit to take any action the absence of which, could reasonably be expected to prevent the satisfaction of the condition set forth in Section 6.2(a).



**5.2** Negative Covenant of Buyer. During the Pre-Closing Period, except as otherwise expressly contemplated by this Agreement or with Seller's prior written consent, which consent shall not be unreasonably withheld or delayed, Buyer shall not, and shall cause its Affiliates not to, knowingly take or permit any action that, or omit to take any action the absence of which, could reasonably be expected to prevent the satisfaction of the condition set forth in Section 6.1(a).

**5.3** Compliance with Legal Requirements. During the Pre-Closing Period, and from and after the Closing, Seller shall, and shall cause its Affiliates and its and their respective successors in interest to the rare pediatric disease product for which the Priority Review Voucher was awarded to, comply with all Legal Requirements applicable to such Persons relating to the Priority Review Voucher and to forward to Buyer any communications it receives from FDA in respect of the Priority Review Voucher. Without limiting the generality of the immediately preceding sentence, to the extent required, now or in the future, under applicable Legal Requirements or otherwise by FDA for the use or transfer of the Priority Review Voucher, or to avoid revocation of the Priority Review Voucher, Seller shall, and shall cause its Affiliates and its and their respective successors in interest to the rare pediatric disease product for which the Priority Review Voucher was awarded to, submit a post-approval production report to the United States Secretary of Health and Human Services not later than five (5) years after the approval of such rare pediatric disease product in accordance with section 529(e)(2) of the FDCA.

**5.4** No Solicitation.

(a) During the Pre-Closing Period, Seller shall not, nor shall it authorize, instruct or permit any of its Affiliates or its or their respective officers, directors or employees or any investment banker, attorney or other advisor or representative retained by it, to (i) solicit, initiate, facilitate or knowingly encourage any inquiries, proposals or offers with respect to, or the submission of, any Alternative Transaction by any Person (other than Buyer or its Affiliates or representatives) or any inquiry, proposal or offer that is reasonably likely to lead to an Alternative Transaction, (ii) engage, continue or participate in any discussions or negotiations regarding, or take any other action intended or reasonably expected to facilitate the making of any inquiry or proposal to Seller that constitutes, or may reasonably be expected to lead to, any Alternative Transaction by any Person (other than Buyer or its Affiliates or their respective representatives) other than to state that they are not permitted to have discussions and to refer to this Agreement, (iii) accept any proposal or offer from any Person (other than Buyer) in respect of an Alternative Transaction, or (iv) resolve to propose or agree to do any of the foregoing.

(b) Upon execution of this Agreement, Seller and its Affiliates shall immediately cease and cause to be terminated any existing discussions with any Person (other than Buyer) that are in respect of an Alternative Transaction.

## 5.5 Antitrust Notification.

(a) Unless this Agreement shall have been validly terminated in accordance with Section 7.1, Buyer and Seller shall, as promptly as practicable after the Effective Date, but no later than the end of the tenth (10<sup>th</sup>) Business Day following the Effective Date, (i) file with the Federal Trade Commission (the “*FTC*”) and the Antitrust Division of the Department of Justice (the “*DOJ*”) the premerger notification and report form required as a result of the contemplated purchase and sale of the Purchased Assets and the other transactions contemplated hereby, and shall include any supplemental information requested in connection therewith, pursuant to the HSR Act, and (ii) make such other filings as are necessary or advisable in other jurisdictions in order to comply with all applicable Legal Requirements relating to competition, merger control or antitrust and shall promptly provide any supplemental information requested by applicable Governmental Entities relating thereto. Any such filing, notification and report form and supplemental information shall be in substantial compliance with the requirements of the HSR Act or such other applicable Legal Requirement. The Parties shall work together and shall furnish to one another such necessary information and reasonable assistance as the other may request in connection with its preparation of any filing or submission which is necessary under the HSR Act or such other applicable Legal Requirement. The Parties shall (A) cooperate with one another and keep one another apprised of the status of any communications with, and any inquiries or requests for additional information from, the FTC, the DOJ or any other applicable Governmental Entity, (B) comply promptly with any such reasonable inquiry or request, (C) not participate, or permit its Affiliates to participate, in any substantive meeting or discussion with any Governmental Entity in respect of any filings, investigation or inquiry concerning this Agreement unless it consults with the other Party in advance and, to the extent permitted by such Governmental Entity, gives the other Party the opportunity to attend and participate thereat, and (D) with the exception of business documents deemed highly confidential by the possessing Party (including documents submitted as attachments to the Party’s notification and report form under the HSR Act), and subject to any other applicable confidentiality restrictions or restrictions required by applicable Legal Requirements, furnish the other Party or the other Party’s outside counsel with copies of all correspondence, filings, and communications (and memoranda setting forth the substance thereof) between a Party or its Affiliates, on the one hand, and any Governmental Entity, on the other hand, with respect to the transactions contemplated hereunder or any investigation with respect to the transactions contemplated hereunder. In addition, to the extent reasonably practicable, all discussions, telephone calls, and meetings with a Governmental Entity regarding the transaction contemplated by this Agreement, shall include representatives of both Parties. In this connection and subject to applicable Legal Requirements, the Parties will consult and cooperate with each other in connection with any analyses, appearances, presentations, memoranda, briefs, arguments, and proposals made or submitted to any Governmental Entity regarding the transaction contemplated by this Agreement, including the Asset Purchase, by or on behalf of any Party. Buyer shall pay all filing fees and other charges for the filing under the HSR Act by both Parties.

(b) From and after the date on which the filings are made pursuant to Section 5.5(a), Buyer and Seller shall use reasonable best efforts to obtain any clearance required under the HSR Act or such other applicable Legal Requirement for the purchase and sale of the Purchased Assets and the other transactions contemplated hereby (any such clearance, an “*Antitrust Approval*”), including replying at the earliest practicable date to any requests for information received from the FTC or DOJ pursuant to the HSR Act and making any permitted request for early expiration or termination of the applicable waiting periods under the HSR Act as soon as possible. For purposes of this Section 5.5(b) and of Section 5.5(a), and notwithstanding the foregoing, Buyer shall not under any circumstances be required to agree to (i) any prohibition, limitation or any condition on the ability of Buyer or any of its Affiliates to acquire or hold, or exercise full rights of ownership or use of, the Purchased Assets, including the use of the Purchased Assets to obtain Priority Review of a product candidate of Buyer or any other benefit associated with the Purchased Assets, (ii) any requirement to dispose of or hold separate any portion of the business or assets of Buyer or its Affiliates, in each case as a result of the purchase and sale of the Purchased Assets or any of the other transactions contemplated by this Agreement, or (iii) any material modification or waiver of the terms and conditions of this Agreement.

(c) Notwithstanding the foregoing, nothing in this Agreement shall require, or be construed to require, the Seller to offer or agree to (A) (i) sell, hold, hold separate, divest, license, discontinue or limit, before or after the Closing Date, any assets, businesses, equity holdings, intellectual property, or other interests or (ii) any conditions relating to, or changes or restrictions in, the operations of any such assets, businesses, equity holdings, intellectual property or interests (including but not limited to any requirements to enter into new contracts or modify or terminate existing Contracts) or (B) any material modification or waiver of the terms and conditions of this Agreement.

**5.6** Expenses. Whether or not the purchase and sale of the Purchased Assets and the other transactions contemplated by this Agreement are consummated, and except as otherwise set forth in this Agreement, each of the Parties shall bear its own fees and expenses incurred or owed in connection with the purchase and sale of the Purchased Assets, this Agreement and the transactions contemplated hereby.

**5.7** Further Assurances. During the Pre-Closing Period, and from and after the Closing, the Parties shall cooperate reasonably with each other in connection with any steps required to be taken as part of their respective obligations under this Agreement, including without limitation any notifications or filings required to be made to the FDA in connection with the transfer of the Purchased Assets (such as the Pending Transfer Notification), and shall (a) furnish upon request to each other such further information, (b) execute and deliver to each other such other documents, and (c) do such other acts and things, all as the other Party may reasonably request for the purpose of carrying out the intent of this Agreement and the transactions contemplated by this Agreement, including the use of the Purchased Assets to obtain Priority Review.

**5.8** Confidentiality. Each Party shall, and shall cause its Affiliates and its and their respective officers, directors, employees and agents to, keep confidential and not publish or otherwise disclose to a third party and not use, directly or indirectly, for any purpose, any Confidential Information furnished or otherwise made known to it, directly or indirectly, by the other Party, except to the extent such disclosure or use is expressly permitted by the terms of this Agreement. In addition, with respect to Confidential Information received from a Party, the other Party will (i) not use such Confidential Information for any reason other than to carry out the intent and purpose of this Agreement, and (ii) not disclose such Confidential Information to any Person, except in each case as otherwise expressly permitted by this Agreement or with the prior written consent of the disclosing Party. Notwithstanding the foregoing, to the extent the receiving Party can demonstrate by documentation or other competent proof, the confidentiality and non-use obligations under this Section 5.8 with respect to any Confidential Information shall not include any information that:

(a) has been published by a third party or otherwise is or hereafter becomes part of the public domain by public use, publication, general knowledge or the like through no wrongful act, fault or negligence on the part of the receiving Party;

(b) has been in the receiving Party's possession prior to disclosure by the disclosing Party without any obligation of confidentiality with respect to such information;

(c) is subsequently received by the receiving Party from a third party without restriction and without breach of any agreement between such third party and the disclosing Party;

(d) is generally made available to third parties by the disclosing Party without restriction on disclosure; or

(e) has been independently developed by or for the receiving Party without reference to, or use or disclosure of, the disclosing Party's Confidential Information.

Specific aspects or details of Confidential Information shall not be deemed to be within the public domain or in the possession of the receiving Party merely because the Confidential Information is embraced by more general information in the public domain or in the possession of the receiving Party. Further, any combination of Confidential Information shall not be considered in the public domain or in the possession of the receiving Party merely because individual elements of such Confidential Information are in the public domain or in the possession of the receiving Party unless the combination and its principles are in the public domain or in the possession of the receiving Party.

**5.9** Permitted Disclosures. Each Party may disclose Confidential Information to the extent that such disclosure is:

(a) in the reasonable opinion of the receiving Party's legal counsel, required to be disclosed pursuant to Law, regulation or a valid order of a court of competent jurisdiction or other Governmental Entity of competent jurisdiction (including by reason of filing with securities regulators, but subject to Section 5.10); *provided*, that the receiving Party shall first have given prompt written notice (and to the extent possible, at least five (5) Business Days' notice) to the disclosing Party and given the disclosing Party a reasonable opportunity to take whatever action it deems necessary to protect its Confidential Information (for example, quash such order or to obtain a protective order or confidential treatment requiring that the Confidential Information and documents that are the subject of such order be held in confidence by such court or Governmental Entity or, if disclosed, be used only for the purposes for which the order was issued). In the event that no protective order or other remedy is obtained, or the disclosing Party waives compliance with the terms of this Agreement, the receiving Party shall furnish only that portion of Confidential Information which the receiving Party is advised by counsel is legally required to be disclosed;

(b) made to its or its Affiliates' financial and legal advisors who have a need to know such disclosing Party's Confidential Information and are either under professional codes of conduct giving rise to expectations of confidentiality and non-use or under written agreements of confidentiality and non-use, in each case, at least as restrictive as those set forth in this Agreement; *provided*, that the receiving Party shall remain responsible for any failure by such financial and legal advisors, to treat such Confidential Information as required under this Article;

(c) made by or on behalf of Buyer or its Affiliates to the FDA or other Governmental Entity as required in connection with any filing, application or request for regulatory approval in which Priority Review is sought; or

(d) made by Buyer or its Affiliates to its or their advisors, consultants, clinicians, vendors, service providers, contractors, existing or prospective collaboration partners, licensees, sublicensees, or other third parties as may be necessary or useful in connection with the transactions contemplated by this Agreement, including the use of the Purchased Assets to obtain Priority Review; *provided*, that such Persons shall be subject to obligations of confidentiality and non-use with respect to such Confidential Information substantially similar to (and at least as restrictive as those set forth in this Agreement) the obligations of confidentiality and non-use of the receiving Party pursuant to Section 5.8.

**5.10** Public Announcements. Seller and Buyer shall consult with each other before issuing, and provide each other the opportunity to review and comment upon, any press release or other public statement with respect to the transactions contemplated hereby, and shall not issue any such press release or make any such public statement prior to such consultation, except as may be required by applicable Law. Buyer and Seller each acknowledges that the other Party, as a publicly traded company is legally obligated to make timely disclosures of materials events relating to its business. The Parties acknowledge that either or both Parties may be obligated to file a copy of this Agreement with the United States Securities and Exchange Commission. Without limiting the foregoing, any Party so obligated shall provide the other Party with a reasonable opportunity to review, comment and request confidential treatment of this Agreement pursuant to applicable rules under the Securities Exchange Act of 1934, as amended, and the Freedom of Information Act and the rules promulgated thereunder to permit the filing of a redacted exhibit. Notwithstanding the foregoing, without prior submission to or approval of the other Party, either Party may issue press releases or public announcements which incorporate information concerning this Agreement which information was included in a press release or public disclosure which was previously disclosed under the terms of this Agreement.

**5.11** Use of Name. Except as expressly provided herein, neither Party shall mention or otherwise use the name, logo, or trademark of the other Party or any of its Affiliates (or any abbreviation or adaptation thereof) in any publication, press release, marketing and promotional material, or other form of publicity without the prior written approval of such other Party in each instance. The restrictions imposed by this Section 5.11 shall not prohibit either Party from making any disclosure identifying the other Party that, in the opinion of the disclosing Party's counsel, is required by applicable Law or the rules of a stock exchange on which the securities of the disclosing Party are listed; *provided*, that such Party shall submit the proposed disclosure identifying the other Party in writing to the other Party as far in advance as reasonably practicable (and in no event less than two (2) Business Days prior to the anticipated date of disclosure) so as to provide a reasonable opportunity to comment thereon.

**5.12** FDA Pending Transfer Notification. Within one (1) Business Day following the Effective Date, Buyer and Seller will jointly submit the notification of Buyer's intent to redeem the Priority Review Voucher, in substantially the form attached hereto as Exhibit 5.12 (the "Pending Transfer Notification").

## ARTICLE 6 CONDITIONS PRECEDENT

**6.1** Conditions Precedent to Seller's Obligation. The obligation of Seller to consummate the transactions contemplated by this Agreement is subject to the satisfaction or waiver on or prior to the Closing Date of the following conditions:

(a) Representations and Warranties of Buyer. Each of the representations and warranties of Buyer made in ARTICLE 4 shall be true and correct in all respects as of the Effective Date and as of the Closing Date, unless the failure of such representations to be so true and correct would not, individually or in the aggregate, have a material adverse effect on the ability of Buyer to consummate the transactions contemplated hereby.

(b) Compliance with Agreements and Covenants. Buyer and its Affiliates shall have complied in all material respects with all covenants, obligations and agreements of Buyer to be performed or complied with by Buyer or any such Affiliate on or before the Closing Date.

(c) Antitrust. Any waiting period (or extension thereof) applicable to the transactions contemplated by this Agreement under the HSR Act shall have been terminated or shall have expired.

(d) No Injunction or Legal Restraint. No Governmental Entity shall have enacted, issued, promulgated, enforced or entered any Law nor shall any temporary restraining order, preliminary or permanent injunction or other order or decree have been issued by any court of competent jurisdiction (other than any such orders, injunctions or decrees issued due to any Action commenced by or on behalf of Seller) that is in effect and which has the effect of making the transactions contemplated by this Agreement illegal or otherwise preventing, prohibiting or restraining the consummation of the transactions contemplated by this Agreement.

(e) Closing Deliveries. Buyer shall have made the deliveries contemplated under Section 2.6.

**6.2** Conditions Precedent to Buyer's Obligation. The obligation of Buyer to consummate the transactions contemplated by this Agreement is subject to the satisfaction or waiver on or prior to the Closing Date of the following conditions:

(a) Representations and Warranties of Seller. Each of the representations and warranties of Seller made in (i) Sections 3.1, 3.2, 3.3, 3.4, 3.5, 3.7, 3.9, 3.10, 3.11 and 3.12 shall be true and correct in all respects as of the Effective Date and as of the Closing Date, and (ii) Sections 3.6, 3.8, and 3.13 shall be true and correct in all respects, in each case, as of the Effective Date and as of the Closing Date, except where the failure of such representations and warranties to be so true and correct would not, individually or in the aggregate, reasonably be expected to delay, restrict, limit, preclude or otherwise negatively impact in a material manner the transfer and/or use of the Purchased Assets to or by Buyer.

(b) Compliance with Agreements and Covenants. Seller and its Affiliates shall have complied in all material respects with all covenants, obligations and agreements of Seller to be performed or complied with by Seller or any such Affiliate on or before the Closing Date.

(c) Antitrust. Any waiting period (or extension thereof) applicable to the transactions contemplated by this Agreement under the HSR Act shall have been terminated or shall have expired.

(d) No Injunction or Legal Restraint. No Governmental Entity shall have enacted, issued, promulgated, enforced or entered any Law nor shall any temporary restraining order, preliminary or permanent injunction or other order or decree have been issued by any court of competent jurisdiction (other than any such orders, injunctions or decrees issued due to any Action commenced by or on behalf of Buyer) that is in effect and which has the effect of making the transactions contemplated by this Agreement illegal or otherwise preventing, prohibiting or restraining the consummation of the transactions contemplated by this Agreement.

(e) Closing Deliveries. Seller shall have made the deliveries contemplated under Section 2.5.

(f) Pending Transfer Notification. The Pending Transfer Notification shall not have been withdrawn, rescinded, modified or amended by Seller.

## ARTICLE 7 TERMINATION

**7.1** Termination. This Agreement may be terminated, and the transactions contemplated hereby may be abandoned, at any time prior to Closing:

(a) by mutual written consent of Buyer and Seller;

(b) by Buyer or Seller, if the Closing has not occurred by the close of business on March 15, 2021; provided that that the right to terminate this Agreement under this Section 7.1(b) shall not be available to any Party whose material breach of any provision set forth in this Agreement has resulted in the failure of the Closing to occur on or before such date

(c) by Buyer or Seller, if (i) any Law having the effect referred to in Section 6.2(d) or Section 6.1(d), as applicable, has been enacted, issued, promulgated, enforced or entered, or (ii) any order, injunction or decree having the effect referred to in Section 6.2(d) or Section 6.1(d), as applicable, is in effect and has become final and nonappealable;

(d) by Buyer, if there has been a violation or breach by Seller of any of its representations, warranties, covenants or other agreements contained in this Agreement, which has prevented or would prevent the satisfaction of any condition to the obligations of Buyer at the Closing, and (i) such violation or breach has not been waived by Buyer, (ii) Buyer has provided written notice to Seller of such violation or breach setting forth the allegations of violation or breach in reasonable detail, and (iii) such violation or breach cannot be or has not been cured by Seller within twenty (20) Business Days after receiving written notice thereof from Buyer; or

(e) by Seller, if there has been a violation or breach by Buyer of any of its representations, warranties, covenants or other agreements contained in this Agreement, which has prevented or would prevent the satisfaction of any condition to the obligations of Seller at the Closing and (i) such violation or breach has not been waived by Seller, (ii) Seller has provided written notice to Buyer of such violation or breach setting forth the allegations of violation or breach in reasonable detail, and (iii) such violation or breach cannot be or has not been cured by Buyer within twenty (20) Business Days after receiving written notice thereof from Seller.

**7.2** Effect of Termination. If this Agreement is terminated and the transactions contemplated hereby are abandoned as described in this ARTICLE 7, this Agreement shall become void and of no further force or effect, except that the provisions of this Section 7.2, Sections 5.8 5.8, 5.9 and 5.10, and ARTICLE 1 and ARTICLE 9 shall survive the termination of this Agreement and shall remain in full force and effect; *provided*, that nothing in this Section 7.2 shall be deemed to release any Party from any liability in respect of any breach by such Party of the terms and provisions of this Agreement prior to the effective date of such termination.

**ARTICLE 8**  
**INDEMNIFICATION**

**8.1**     Indemnification.

(a) Indemnification by Seller. From and after the Closing, Seller shall indemnify, defend and hold Buyer and its Affiliates and its and their respective directors, officers, employees, shareholders, partners, members and agents (each, a “**Buyer Indemnified Party**”) harmless for, from and against any and all Losses which any Buyer Indemnified Party may suffer, incur, sustain or become subject to, arising out of or directly or indirectly resulting from (i) any breach or violation of or inaccuracy in any representations or warranties of Seller under this Agreement, (ii) any breach or violation of any covenant or agreement of Seller under this Agreement, or (iii) Seller’s grossly negligent or wrongful acts, omissions or misrepresentations, regardless of the form of action, in connection with this Agreement.

(b) Indemnification by Buyer. From and after the Closing, Buyer shall indemnify, defend and hold Seller and its Affiliates, and its and their respective directors, officers, employees, shareholders, partners, members and agents (each, a “**Seller Indemnified Party**”) harmless for, from and against any and all Losses which any Seller Indemnified Party may suffer, incur, sustain or become subject to, arising out of or directly or indirectly resulting from (i) any breach or violation of or inaccuracy in any representations or warranties of Buyer under this Agreement, (ii) any breach or violation of any covenant or agreement of Buyer under this Agreement, (iii) Buyer’s grossly negligent or wrongful acts, omissions or misrepresentations, regardless of the form of action, in connection with this Agreement, or (iv) Buyer’s, its Affiliates’ or any subsequent transferee’s use or ownership of the Purchased Assets.

**8.2**     Notice of Loss; Third Party Claims.

(a) A claim for indemnification for any matter not involving a Third Party Claim may be asserted by written notice to the Party from whom indemnification is sought. Such notice shall include the facts constituting the basis for such claim for indemnification, the Sections of this Agreement upon which such claim for indemnification is then based and an estimate, if possible, of the amount of Losses suffered or reasonably expected to be suffered by the Indemnified Party.



(b) In the event that any claim shall be instituted or asserted by any third party in respect of which payment may be sought under Section 8.1(a) or Section 8.1(b) hereof (each, a “**Third Party Claim**”), the Indemnified Party shall promptly cause written notice of the assertion of any Third Party Claim of which it has knowledge which is covered by the provisions of Section 8.1(a) or Section 8.1(b), as applicable, to be forwarded to the Indemnifying Party. The failure of the Indemnified Party to give reasonably prompt notice of any Third Party Claim shall not release, waive or otherwise affect the Indemnifying Party’s obligations with respect thereto except to the extent that the Indemnifying Party is actually prejudiced as a result of such failure. The Indemnifying Party shall have the right, at its sole option and expense, to be represented by counsel reasonably acceptable to the Indemnified Party and to defend against, negotiate, settle or otherwise deal with any Third Party Claim which relates to any Losses indemnified by it hereunder, subject to the provisions below; *provided, however*, that the Indemnifying Party may not assume control of defense to a Third Party Claim (i) in which equitable relief other than monetary damages is sought, or (ii) if the Indemnifying Party has not notified the Indemnified Party in writing that it will be liable to indemnify the Indemnified Party with respect to all Losses relating to such Third Party Claim. If the Indemnifying Party elects to defend against, negotiate, settle or otherwise deal with any Third Party Claim which relates to any Losses indemnified by it hereunder, it shall within thirty (30) days (or sooner, if the nature of the Third Party Claim so requires) notify the Indemnified Party of its intent to do so. If the Indemnifying Party elects not to defend against, negotiate, settle or otherwise deal with any Third Party Claim which relates to any Losses indemnified against hereunder, or is not permitted to assume the defense of a Third Party Claim pursuant to the proviso to the third sentence of this Section 8.2(b), the Indemnified Party may defend against, negotiate, settle or otherwise deal with such Third Party Claim, subject to the provisions below. If the Indemnifying Party shall assume the defense of any Third Party Claim pursuant to the terms of this Agreement, the Indemnified Party may participate, at his or its own expense, in the defense of such Third Party Claim; *provided, however*, that such Indemnified Party shall be entitled to participate in any such defense with separate counsel at the expense of the Indemnifying Party if (A) so requested by the Indemnifying Party to participate or (B) in the reasonable opinion of outside counsel to the Indemnified Party a conflict or potential conflict exists between the Indemnified Party and the Indemnifying Party that would make such separate representation advisable. The Parties hereto agree to reasonably cooperate with each other in connection with the defense, negotiation or settlement of any such Third Party Claim. Notwithstanding anything in this Section 8.2 to the contrary, the Indemnifying Party shall not, without the written consent of the Indemnified Party, settle or compromise any Third Party Claim or permit a default or consent to entry of any judgment unless (1) the claimant provides to the Indemnified Party an unqualified release of the Indemnified Parties from all liability in respect of such Third Party Claim, (2) such settlement does not involve any injunctive relief binding upon the Indemnified Party or any of its Affiliates, (3) such settlement does not encumber any of the material assets of any Indemnified Party or impose any restriction or condition that would apply to or materially affect any Indemnified Party or the conduct of any Indemnified Party’s business, and (4) such settlement does not involve any admission of liability or wrongdoing by any Indemnified Party or any of its Affiliates.

(c) In the event that the Indemnified Party conducts the defense of the Third Party Claim pursuant to this Section 8.2, the Indemnifying Party will (i) advance the Indemnified Party promptly and periodically for the reasonable costs of defending against the Third Party Claim (including reasonable attorneys’ and experts’ fees and expenses) and (ii) remain responsible for any and all other Losses that the Indemnified Party may incur or suffer resulting from, arising out of, relating to, in the nature of or caused by the Third Party Claim to the fullest extent provided in this ARTICLE 8.

**8.3** Direct Claims. A claim for indemnification for any matter not involving a Third Party Claim may be asserted by written notice to the Party from whom indemnification is sought. Such notice shall include the facts constituting the basis for such claim for indemnification, the Sections of this Agreement upon which such claim for indemnification is then based, and an estimate, if possible, of the amount of Damages suffered or reasonably expected to be suffered by the Indemnitee.

**8.4** Adjustments. Any amount paid under this Article VIII shall be treated as an adjustment to the Purchase Price for all tax purposes unless otherwise required by applicable Legal Requirements.

**8.5** Survival. All covenants, representations and warranties of the Parties under this Agreement shall survive the execution and delivery of this Agreement and the Closing, as applicable, and (except with respect to claims based on breach of covenant or breach of any of the representations and warranties set forth in Section 3.1, 3.2, 3.3, 3.4, 3.5, 3.10, 3.11, 3.12, 3.13, 4.1, 4.2, 4.3, 4.4 or 4.5, all of which shall survive until the expiration of the applicable statutes of limitations) claims based upon a breach of such representations and warranties will expire at 11:59 p.m. eastern time on the date that is two (2) years after the Closing Date.

## **ARTICLE 9 GENERAL PROVISIONS**

**9.1** Notice Requirements. Any notice, request, demand, waiver, consent, approval, or other communication permitted or required under this Agreement shall be in writing, shall refer specifically to this Agreement and shall be deemed given only if (a) delivered by hand, (b) by email of a PDF attachment (with transmission confirmed), or (c) by internationally recognized overnight delivery service that maintains records of delivery, addressed to the Parties at their respective addresses specified in this Section 9.1 or to such other address as the Party to whom notice is to be given may have provided to the other Party in accordance with this Section 9.1. Such notice shall be deemed to have been given as of the date delivered by hand or transmitted by email of a PDF attachment (with transmission confirmed) or on the second Business Day (at the place of delivery) after deposit with an internationally recognized overnight delivery service. Any notice delivered by email of a PDF attachment shall be confirmed by a hard copy delivered as soon as practicable thereafter.

If to Buyer, to:

United Therapeutics Corporation  
1735 Connecticut Avenue, NW  
Washington, D.C. 20016  
Attention: General Counsel  
E-mail to: [legal@unither.com](mailto:legal@unither.com)

If to Seller, to:

Y-mAbs Therapeutics, Inc.  
230 Park Avenue, Suite 3350  
New York, NY 10169  
Attention: Thomas Gad  
E-mail to: [Tgad@ymabs.com](mailto:Tgad@ymabs.com) and to [Srn@ymabs.com](mailto:Srn@ymabs.com)

**9.2** Priority Review Fee. The priority review fee and all other user fees under the FDCA applicable to the human drug application for which the Priority Review Voucher is redeemed shall be borne exclusively by Buyer or any transferee of the Priority Review Voucher. In any event, Seller shall have no liability or obligation for any such fees.

**9.3** Construction. Except where the context otherwise requires, wherever used, the singular shall include the plural, the plural the singular, the use of any gender shall be applicable to all genders and the word “or” is used in the inclusive sense (and/or). Whenever this Agreement refers to a number of days, unless otherwise specified, such number refers to calendar days. The captions of this Agreement are for convenience of reference only and in no way define, describe, extend, or limit the scope or intent of this Agreement or the intent of any provision contained in this Agreement. The term “including,” “include,” or “includes” as used herein shall mean “including, but not limited to,” and shall not limit the generality of any description preceding such term. The language of this Agreement shall be deemed to be the language mutually chosen by the Parties and no rule of strict construction shall be applied against either Party hereto. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

**9.4** References. Unless otherwise specified, (a) references in this Agreement to any Article, Section, Schedule or Exhibit shall mean references to such Article, Section, Schedule or Exhibit of this Agreement, (b) references in any Section to any clause are references to such clause of such Section, and (c) references to any agreement, instrument, or other document in this Agreement refer to such agreement, instrument, or other document as originally executed or, if subsequently amended, replaced, or supplemented from time to time, as so amended, replaced, or supplemented and in effect at the relevant time of reference thereto.

**9.5** Entire Agreement; Amendments. This Agreement sets forth and constitutes the entire agreement and understanding between the Parties with respect to the subject matter hereof and all prior agreements, understandings, promises, and representations, whether written or oral, with respect thereto are superseded hereby (including that certain Non-Binding Proposal to Acquire Rare Pediatric Disease Priority Review Voucher, dated December 18, 2020). Each Party confirms that it is not relying on any representations or warranties of the other Party except as specifically set forth in this Agreement. No amendment, modification, release, or discharge shall be binding upon the Parties unless in writing and duly executed by authorized representatives of both Parties.

**9.6** Assignment. Without the prior written consent of the other Party neither Party shall sell, transfer, assign, delegate, pledge, or otherwise dispose of, whether voluntarily, involuntarily, by operation of law or otherwise, this Agreement or any of its rights or duties hereunder; *provided*, that (a) either Party may make such an assignment without the other Party’s consent to any of its Affiliates or to a successor, whether in a merger, sale of stock, sale of all or substantially all assets or any other similar transaction, and (b) Buyer may make such an assignment, in whole or in part, without Seller’s consent, to any purchaser, transferee, or assignee of the Purchased Assets. With respect to any permitted assignment, the assigning Party shall remain responsible for the performance by such permitted assignee of the assigning Party’s duties and obligations hereunder. Any attempted assignment or delegation in violation of this Section 9.6 shall be void and of no effect. All validly assigned and delegated rights and obligations of the Parties hereunder shall be binding upon and inure to the benefit of and be enforceable by and against the successors and permitted assigns of Buyer or Seller, as the case may be. The permitted assignee or transferee shall assume all obligations of its assignor or transferor under this Agreement.

**9.7** Severability. If any provision of this Agreement is held to be illegal, invalid, or unenforceable under any present or future law, and if the rights or obligations of either Party under this Agreement will not be materially and adversely affected thereby, (a) such provision shall be fully severable, (b) this Agreement shall be construed and enforced as if such illegal, invalid, or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement shall remain in full force and effect and shall not be affected by the illegal, invalid, or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid, or unenforceable provision, there shall be added automatically as a part of this Agreement a legal, valid, and enforceable provision as similar in terms to such illegal, invalid, or unenforceable provision as may be possible and reasonably acceptable to the Parties. To the fullest extent permitted by applicable law, each Party hereby waives any provision of law that would render any provision hereof illegal, invalid, or unenforceable in any respect.

**9.8** Governing Law. This Agreement or the performance, enforcement, breach or termination hereof shall be interpreted, governed by and construed in accordance with the laws of the State of Delaware, United States, excluding any conflicts or choice of law rule or principle that might otherwise refer construction or interpretation of this Agreement to the substantive law of another jurisdiction.

**9.9** Submission to Jurisdiction. Each Party irrevocably agrees that any legal action or proceeding arising out of or relating to this Agreement brought by the other Party or its successors or assigns shall be brought and determined in any Delaware state or federal court, and each of the Parties hereby irrevocably submits to the exclusive jurisdiction of the aforesaid courts with regard to any such action or proceeding arising out of or relating to this Agreement and the transactions contemplated hereby. Each Party agrees not to commence any action, suit or proceeding relating thereto except in the courts described above in Delaware, other than actions in any court of competent jurisdiction to enforce any judgment, decree or award rendered by any such court in Delaware as described herein. Each Party hereby irrevocably and unconditionally waives, and agrees not to assert, by way of motion or as a defense, counterclaim or otherwise, in any action or proceeding arising out of or relating to this Agreement or the transactions contemplated hereby, (a) any claim that it is not personally subject to the jurisdiction of the courts in Delaware as described herein for any reason, (b) that it or its property is exempt or immune from jurisdiction of any such court or from any legal process commenced in such courts (whether through service of notice, attachment prior to judgment, attachment in aid of execution of judgment, execution of judgment or otherwise) and (c) that (i) the suit, action or proceeding in any such court is brought in an inconvenient forum, (ii) the venue of such suit, action or proceeding is improper or (iii) this Agreement, or the subject matter hereof, may not be enforced in or by such courts.

**9.10** WAIVER OF JURY TRIAL. EACH PARTY, TO THE EXTENT PERMITTED BY LAW, KNOWINGLY, VOLUNTARILY, AND INTENTIONALLY WAIVES ITS RIGHT TO A TRIAL BY JURY IN ANY ACTION OR OTHER LEGAL PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT AND THE TRANSACTIONS IT CONTEMPLATES. THIS WAIVER APPLIES TO ANY ACTION OR LEGAL PROCEEDING, WHETHER SOUNDING IN CONTRACT, TORT, OR OTHERWISE.

**9.11** Waiver and Non-Exclusion of Remedies. Any term or condition of this Agreement may be waived at any time by the Party that is entitled to the benefit thereof, but no such waiver shall be effective unless set forth in a written instrument duly executed by or on behalf of the Party waiving such term or condition. The waiver by either Party hereto of any right hereunder or of the failure to perform or of a breach by the other Party shall not be deemed a waiver of any other right hereunder or of any other breach or failure by such other Party whether of a similar nature or otherwise. The rights and remedies provided herein are cumulative and do not exclude any other right or remedy provided by applicable law or otherwise available except as expressly set forth herein.

**9.12** No Benefit to Third Parties. Except as provided in ARTICLE 8, covenants and agreements set forth in this Agreement are for the sole benefit of the Parties hereto and their successors and permitted assigns, and they shall not be construed as conferring any rights on any other Persons.

**9.13** Counterparts; Facsimile Execution. This Agreement may be executed in two (2) or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one (1) and the same instrument. This Agreement may be executed by facsimile or electronically transmitted signatures and such signatures shall be deemed to bind each Party hereto as if they were original signatures.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, each of Buyer and Seller has caused this Asset Purchase Agreement to be executed and delivered by their respective officers thereunto duly authorized, all as of the date first written above.

**UNITED THERAPEUTICS CORPORATION**

By: \_\_\_\_\_

Name: Michael Benkowitz

Title: President and Chief Operating Officer

**Y-MABS THERAPEUTICS, INC.**

By: \_\_\_\_\_

Name: Thomas Gad

Title: President

By: \_\_\_\_\_

Name: Claus Møller

Title: Chief Executive Officer

[Signature Page to Asset Purchase Agreement]

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**Exhibit 2.5(a)**

**Form of Seller FDA Transfer Notification**

[Y-mAbs Therapeutics, Inc. Letterhead]

[Date]

Harpreet Singh, M.D., Division Director (Acting)  
Division of Oncology 2  
Office of Oncologic Diseases  
Center for Drug Evaluation and Research  
Food and Drug Administration  
5901-B Ammendale Road  
Beltsville, MD 20705-1266

**Re: Transfer of Rare Pediatric Disease Priority Review Voucher No. PRV BLA 761171**

Dear Dr. Singh:

Reference is made to the approval letter dated November 25, 2020 from the U.S. Food and Drug Administration (“**FDA**”) to Y-mAbs Therapeutics, Inc. (“**Y-mAbs**”) for biologics license application (“**BLA**”) 761171. In this letter, FDA approved DANYELZA<sup>®</sup> (naxitamab-ggqk) for use with granulocyte-macrophage colony-stimulating factor (GM-CSF), for the treatment of pediatric patients 1 year of age or older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated partial response, minor response, or stable disease prior to therapy. In the letter, FDA also granted Y-mAbs a Rare Pediatric Disease Priority Review Voucher with the tracking number PRV BLA 761171 (the “**Voucher**”). A copy of the approval letter is enclosed. Y-mAbs is the original recipient of the Voucher and has not previously transferred the Voucher.

Further reference is made to the Asset Purchase Agreement (the “**Agreement**”), dated December 24, 2020, between United Therapeutics Corporation, a Delaware corporation (“**UT**”) and Y-mAbs, a Delaware corporation. Pursuant to the Agreement, Y-mAbs has sold, transferred, assigned, conveyed and delivered the Voucher to UT, and UT has acquired and accepted the Voucher from Y-mAbs, effective \_\_\_\_\_, 2021. This transfer is free and clear of all liens and provides UT will all of Y-mAbs’ rights, titles and interest in, to, and under, the Voucher.

This letter acknowledges that Y-mAbs has transferred, assigned, conveyed, and delivered the Voucher to UT. This letter will be presented to FDA as evidence that Y-mAbs acknowledges the sale and transfer of the Voucher from Y-mAbs to UT.

Sincerely,

Y-MABS THERAPEUTICS, INC.

By: \_\_\_\_\_

Name: \_\_\_\_\_

Title: \_\_\_\_\_

Enclosure

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**Exhibit 2.5(b)**

**Form of Seller Compliance Certificate**

Y-MABS THERAPEUTICS, INC.

Closing Certificate

\_\_\_\_\_, 2021

This Closing Certificate (this "**Certificate**") is delivered pursuant to Section 2.5(b) of the Asset Purchase Agreement (the "**Agreement**"), dated as of December 24, 2020, by and between United Therapeutics Corporation ("**Buyer**") and Y-mAbs Therapeutics, Inc. ("**Seller**"). Unless otherwise defined herein or if the context otherwise requires, capitalized terms used in this Certificate have the meanings provided in the Agreement.

The undersigned, \_\_\_\_\_, in [his/her] capacity as a duly authorized officer of Seller, solely in such capacity and not in [his/her] individual capacity, is duly authorized to execute and deliver this Certificate on behalf of Seller. By executing this Certificate, the undersigned hereby certifies to Buyer that as of the date hereof:

1. Each of the (i) representations and warranties of Seller set forth in Sections 3.1, 3.2, 3.3, 3.4, 3.5, 3.7, 3.9, 3.10, 3.11 and 3.12 of the Agreement were and are true and correct in all respects as of the Effective Date and as of the date hereof, and (ii) representations and warranties of Seller set forth in Sections 3.6, 3.8 and 3.13 are true and correct in all material respects, in each case, as of the Effective Date and as of the date hereof, except where the failure of such representations and warranties to be so true and correct would not, individually or in the aggregate, reasonably be expected to delay, restrict, limit, preclude or otherwise negatively impact in a material manner the transfer and/or use of the Purchased Assets to or by Buyer.
2. Seller and its Affiliates have complied in all material respects with all covenants, obligations and agreements of Seller required by the Agreement to be performed or complied with by Seller or any such Affiliate on or before the date hereof.

IN WITNESS WHEREOF, the undersigned has executed and delivered this Certificate as of the date first set forth above.

By: \_\_\_\_\_  
Name:  
Title:

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**Exhibit 2.6(b)**

**Form of Buyer FDA Transfer Notification**

[United Therapeutics Corporation Letterhead]

[Date]

Harpreet Singh, M.D., Division Director (Acting)  
Division of Oncology 2  
Office of Oncologic Diseases  
Center for Drug Evaluation and Research  
Food and Drug Administration  
5901-B Ammendale Road  
Beltsville, MD 20705-1266

**Re: Acceptance of Rare Pediatric Disease Priority Review Voucher  
No. PRV BLA 761171**

Dear Dr. Singh:

Reference is made to the approval letter dated November 25, 2020 from the U.S. Food and Drug Administration (“**FDA**”) to Y-mAbs Therapeutics, Inc. (“**Y-mAbs**”) for biologics license application (“**BLA**”) 761171. In this letter, FDA approved DANYELZA<sup>®</sup> (naxitamab-ggqk) for use with granulocyte-macrophage colony-stimulating factor (GM-CSF), for the treatment of pediatric patients 1 year of age or older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated partial response, minor response, or stable disease prior to therapy. In the letter, FDA also granted Y-mAbs a Rare Pediatric Disease Priority Review Voucher with the tracking number PRV BLA 761171 (the “**Voucher**”). A copy of the approval letter is enclosed.

Further reference is made to the Asset Purchase Agreement (the “**Agreement**”), dated December 24, 2020, between United Therapeutics Corporation, a Delaware corporation (“**UT**”) and Y-mAbs, a Delaware corporation. Pursuant to the Agreement, Y-mAbs has sold, transferred, assigned, conveyed and delivered the Voucher to UT, and UT has acquired and accepted the Voucher from Y-mAbs, effective \_\_\_\_\_, 2021. This transfer is free and clear of all liens and provides UT will all of Y-mAbs’ rights, titles and interest in, to, and under, the Voucher.

This letter acknowledges UT’s acquisition and acceptance of the Voucher from Y-mAbs. This letter will be presented to FDA as evidence that UT acknowledges the sale and transfer of the Voucher from Y-mAbs to UT.

Sincerely,

UNITED THERAPEUTICS CORPORATION

By: \_\_\_\_\_

Name: Dean Bunce

Title: EVP, Global Regulatory Affairs

Enclosure

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**Exhibit 2.6(c)**

**Form of Buyer Compliance Certificate**

UNITED THERAPEUTICS CORPORATION

Closing Certificate

\_\_\_\_\_, 2021

This Closing Certificate (this "**Certificate**") is delivered pursuant to Section 2.6(c) of the Asset Purchase Agreement (the "**Agreement**"), dated as of December 24, 2020, by and between United Therapeutics Corporation ("**Buyer**") and Y-mAbs Therapeutics, Inc. ("**Seller**"). Unless otherwise defined herein or if the context otherwise requires, capitalized terms used in this Certificate have the meanings provided in the Agreement.

The undersigned, \_\_\_\_\_, in [his/her] capacity as a duly authorized officer of Buyer, solely in such capacity and not in [his/her] individual capacity, is duly authorized to execute and deliver this Certificate on behalf of Buyer. By executing this Certificate, the undersigned hereby certifies to Seller that as of the date hereof:

1. Each of the representations and warranties of Buyer made in Article 4 of the Agreement were and are true and correct in all respects as of the Effective Date and as of the date hereof, except where the failure of such representations and warranties to be so true and correct would not, individually or in the aggregate, have a material adverse effect on the ability of Buyer to consummate the transactions contemplated by the Agreement.
2. Buyer and its Affiliates have complied in all material respects with all covenants, obligations and agreements of Buyer required by the Agreement to be performed or complied with by Buyer or any such Affiliate on or before the date hereof.

IN WITNESS WHEREOF, the undersigned has executed and delivered this Certificate as of the date first set forth above.

By: \_\_\_\_\_  
Name:  
Title:

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**Exhibit 5.12**

**Form of FDA Pending Transfer Notification**

[United Therapeutics Corporation Letterhead]

**Notification of Intent to Submit an Application with a Rare Pediatric Disease Priority Review Voucher**

December 28, 2020

Norman Stockbridge, M.D., Ph.D., Director Cardiology and Nephrology  
Division of Regulatory Operations for Cardiology, Hematology, Endocrinology, & Nephrology  
Office of Regulatory Operations FDA, CDER (HFD-110)  
5901B Ammendale Road  
Beltsville, Maryland 20705-1266

**Re: NDA 214324**  
**Tyvaso™ DPI (treprostinil) Inhalation Powder**  
**Sequence No: 0001**  
**Joint Notification of United Therapeutics Corp Intent to Submit a New Drug Application (NDA 214324) with a Rare Pediatric Disease Priority Review Voucher**

Dear Dr. Stockbridge:

Pursuant to Section 529(b)(4)(B)(i) of the Federal Food, Drug, and Cosmetic Act, United Therapeutics Corporation (“UTC”) and Y-mAbs Therapeutics, Inc. (“Y-mAbs”) hereby submit notice of UTC’s intent to submit a 505(b)(1) New Drug Application (the “NDA”) for a dry powder inhalation formulation of treprostinil (Tyvaso DPI™) no earlier than April 1, 2021, with the Rare Pediatric Disease Priority Review Voucher PRV BLA 761171 (the “Voucher”). The sponsor of the NDA will be UTC.

The Voucher was issued to Y-mAbs upon approval of BLA 761171 for DANYELZA® (naxitamab-gqgk) injection, as evidenced in the Federal Register (85 Fed. Reg. 78859 (Dec. 7, 2020)). The Voucher has not been transferred since it was issued.

Y-mAbs and UTC have entered into an Asset Purchase Agreement (the “Agreement”), dated December 24, 2020, pursuant to which Y-mAbs will transfer the Voucher to UTC upon closing, following expiration or early termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvement Act of 1976, as amended (“HSR”), and the satisfaction of certain other customary closing conditions. We anticipate closing will occur on or about January 29, 2021, but the precise closing date depends primarily on how quickly the HSR closing condition is satisfied.

Promptly following closing of the Agreement, both UTC and Y-mAbs will submit letters to FDA acknowledging the transfer of the Voucher from Y-mAbs to UTC. UTC discussed this process via e-mail with FDA’s PDUFA User Fee Staff (Peter Chen, RPh, ExecJD) on December 9 and 10, 2020 and was given authorization to proceed with this notice.

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Please do not hesitate to contact either of the undersigned with any questions, at the email addresses and telephone numbers noted below.

Sincerely,

UNITED THERAPEUTICS CORPORATION

Y-MABS THERAPEUTICS, INC.

By: \_\_\_\_\_

Name: Dean Bunce

Title: EVP, Global Regulatory Affairs

E-mail: [insert]

Telephone: [insert]

By: \_\_\_\_\_

Name:

Title:

E-mail: [insert]

Telephone: [insert]

cc: Peter Chen, RPh, ExecJD  
CAPT, U.S. Public Health Service  
Center for Drug Evaluation and Research  
Office of Management  
U.S. Food and Drug Administration  
(via email: [insert])

Harpreet Singh, M.D., Division Director (Acting)  
Division of Oncology 2  
Office of Oncologic Diseases  
Center for Drug Evaluation and Research  
Food and Drug Administration  
5901-B Ammendale Road  
Beltsville, MD 20705-1266  
(via email:[insert])

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## Y-mAbs Announces Proposed Public Offering of Common Stock

Feb 16, 2021

NEW YORK, Feb. 16, 2021 (GLOBE NEWSWIRE) -- Y-mAbs Therapeutics, Inc. (the "Company" or "Y-mAbs") (Nasdaq: YMAB), a commercial stage biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer, today announced the commencement of a registered underwritten public offering of up to \$100 million in shares of its common stock. All shares in the offering are expected to be offered by the Company. In addition, the Company intends to grant the underwriters a 30-day option to purchase up to \$15 million in shares of its common stock.

J.P. Morgan, Morgan Stanley, and BofA Securities are acting as the joint book-running managers for the proposed offering. The offering is subject to market and other customary closing conditions, and there can be no assurance as to whether or when the offering may be completed.

A shelf registration statement relating to the shares of common stock being offered in the public offering described above was filed on Form S-3 (Reg. No. 333-234034) which was declared effective by the Securities and Exchange Commission (SEC) on October 15, 2019. The securities may be offered only by means of a written prospectus, including a prospectus supplement, forming a part of the effective registration statement. A preliminary prospectus supplement and accompanying prospectus relating to the offering will be filed with the SEC and will be available on the SEC's website at [www.sec.gov](http://www.sec.gov). When available, copies of the preliminary prospectus supplement and the accompanying prospectus relating to the offering may also be obtained from: J.P. Morgan, c/o Broadridge Financial Solutions, 1155 Long Island Avenue, Edgewood, NY, 11717, or by telephone at (866) 803-9204; Morgan Stanley & Co. LLC, Attention: Prospectus Department, 180 Varick Street, Second Floor, New York, New York 10014 or BofA Securities, NC1-004-03-43, 200 North College Street, 3rd Floor, Charlotte, NC 28255-0001, Attn: Prospectus Department, or by emailing [dg.prospectus\\_requests@bofa.com](mailto:dg.prospectus_requests@bofa.com).

This press release does not constitute an offer to sell or the solicitation of an offer to buy, nor shall there be any sale of these securities in any state or jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.

### About Y-mAbs

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. The Company has a broad and advanced product pipeline, including one FDA approved product, DANYELZA® (naxitamab-gqgk), which targets tumors that express GD2, and one pivotal-stage product candidate, omburtamab, which targets tumors that express B7-H3.

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## Forward Looking Statements

Statements in this press release about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute “forward-looking statements” within the meaning of The Private Securities Litigation Reform Act of 1995. Such statements include, but are not limited to, statements about our business model and development, commercialization and product distribution plans; current and future clinical and pre-clinical studies and our research and development programs; expectations related to the timing of the initiation and completion of regulatory submissions; regulatory, marketing and reimbursement approvals; rate and degree of market acceptance and clinical utility as well as pricing and reimbursement levels; retaining and hiring key employees; our commercialization, marketing and manufacturing capabilities and strategy; our intellectual property position and strategy; additional product candidates and technologies; collaborations or strategic partnerships and the potential benefits thereof; expectations related to the use of our cash and cash equivalents, and the need for, timing and amount of any future financing transaction; our financial performance, including our estimates regarding revenues, expenses, capital expenditure requirements; developments relating to our competitors and our industry; and other statements that are not historical facts. Words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “hope,” “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. Our product candidates and related technologies are novel approaches to cancer treatment that present significant challenges. Actual results may differ materially from those indicated by such forward-looking statements as a result of various factors, including but not limited to: risks associated with our financial condition and need for additional capital; risks associated with our development work; cost and success of our product development activities and clinical trials; the risks of delay in the timing of our regulatory submissions or failure to receive approval of our drug candidates; the risks related to commercializing any approved pharmaceutical product including the rate and degree of market acceptance of our product candidates; development of our sales and marketing capabilities and risks associated with failure to obtain sufficient reimbursement for our products; the risks related to our dependence on third parties including for conduct of clinical testing and product manufacture; our inability to enter into partnerships; the risks related to government regulation; risks related to market approval, risks associated with protection of our intellectual property rights; risks related to employee matters and managing growth; risks related to our common stock, risks associated with the pandemic caused by the novel coronavirus known as COVID-19 and other risks and uncertainties affecting the Company including those described in the "Risk Factors" section included in our Annual Report on Form 10-K and in our other SEC filings. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company undertakes no obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

“Y-mAbs®” and “DANYELZA®” are registered trademarks of Y-mAbs Therapeutics, Inc.

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Source: Y-mAbs Therapeutics, Inc

## Y-mAbs Announces Pricing of Public Offering of Common Stock

NEW YORK, February 17, 2021 (GLOBE NEWSWIRE) -- Y-mAbs Therapeutics, Inc. (the "Company" or "Y-mAbs") (Nasdaq: YMAB), a commercial-stage biopharmaceutical company focused on the development and commercialization of novel, antibody-based therapeutic products for the treatment of cancer, today announced the pricing of its public offering of 2,439,025 shares of its common stock at a public offering price of \$41.00 per share. The gross proceeds from the offering, before deducting underwriting discounts and commissions and estimated offering expenses, are expected to be approximately \$100,000,025 million. All of the shares are being offered by Y-mAbs. In addition, Y-mAbs has granted the underwriters a 30-day option to purchase up to an additional 365,853 shares of common stock at the public offering price, less the underwriting discounts. The offering is expected to close on February 22, 2021, subject to the satisfaction of customary closing conditions.

J.P. Morgan, Morgan Stanley and BofA Securities are acting as the joint book-running managers for the offering.

The prospectus and prospectus supplement relating to the offering may be obtained, when available, from: J.P. Morgan, c/o Broadridge Financial Solutions, 1155 Long Island Avenue, Edgewood, NY, 11717, or by telephone at (866) 803-9204; Morgan Stanley & Co. LLC, Attention: Prospectus Department, 180 Varick Street, Second Floor, New York, New York 10014 or BofA Securities, NC1-004-03-43, 200 North College Street, 3rd Floor, Charlotte, NC 28255-0001, Attn: Prospectus Department, or by emailing [dg.prospectus\\_requests@bofa.com](mailto:dg.prospectus_requests@bofa.com).

The shares of common stock described above are being offered by Y-mAbs pursuant to its shelf registration statement on Form S-3 (Reg. No. 333-234034), including a base prospectus, that was declared effective by the SEC on October 15, 2019. The securities are being offered only by means of a prospectus supplement and accompanying prospectus forming a part of the effective registration statement. This press release shall not constitute an offer to sell or the solicitation of an offer to buy, nor shall there be any sale of these securities in any state or jurisdiction in which such an offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of any such state or jurisdiction.

### **About Y-mAbs**

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. The Company has a broad and advanced product pipeline, including one FDA approved product, DANYELZA<sup>®</sup> (naxitamab-gqgk), which targets tumors that express GD2, and one pivotal-stage product candidate, omburtamab, which targets tumors that express B7-H3.

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