magenta
THERAPEUTICS

2018 Annual Report

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

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2018

OR

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

For the transition period from _____ to _____

Commission file number: 001-38541

Magenta Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**81-0724163**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**
100 Technology Square Cambridge, Massachusetts	**02139**
(Address of principal executive offices)	**(Zip Code)**

(857) 242-0170

(Registrant's telephone number, including area code)

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

Title of each class	**Name of exchange on which registered**
Common Stock, $0.001 Par Value	**The Nasdaq Global Market**

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

None

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

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

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405 of this chapter) is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☒

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

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

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

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

The aggregate market value of Common Stock held by non-affiliates of the registrant computed by reference to the price of the registrant's Common Stock as of June 29, 2018, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $209.1 million (based on the last reported sale price on the Nasdaq Global Market as of such date). As of February 28, 2019, there were 33,466,526 shares of the registrant's Common Stock, $0.001 par value per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Proxy Statement for its 2019 Annual Meeting of Stockholders, which the registrant intends to file with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2018, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K of Magenta Therapeutics, Inc. (the "Company") contains or incorporates statements that constitute forward-looking statements within the meaning of the federal securities laws. Any express or implied statements that do not relate to historical or current facts or matters are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "may," "will," "could," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "projects," "seeks," "endeavor," "potential," "continue" or the negative of these terms or other comparable terminology. Forward-looking statements appear in a number of places in this Annual Report on Form 10-K and include, but are not limited to, statements about:

- the timing and the success of clinical trials of MGTA-456 and any other product candidates;
- the outcomes of our preclinical studies, including under our C200 program;
- our ability to enroll patients in our clinical trials at the pace that we project;
- whether the results of our trials will be sufficient to support domestic or foreign regulatory approvals for MGTA-456 or any other product candidates we may develop;
- our ability to establish clinical programs moving forward in multiple indications by 2020, with a rapidly advancing portfolio and sustainable platform;
- regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- our ability to obtain, including on an expedited basis, and maintain regulatory approval of MGTA-456 or any other product candidates we may develop;
- the level of expenses related to any of our product candidates or clinical development programs;
- our expectation that our existing capital resources will be sufficient to enable us to fund our planned development of MGTA-456 and any other product candidates we may identify and pursue;
- the benefits of the use of MGTA-456 or any other product candidate, if approved;
- our ability to successfully commercialize MGTA-456 or any other product candidates we may identify and pursue, if approved;
- our ability to successfully find collaborators for E478 or any of our current and future programs and product candidates;
- the rate and degree of market acceptance of MGTA-456 or any other product candidates we may identify and pursue;
- our ability to obtain orphan drug designation for any of our product candidates we may identify;
- our expectations regarding government and third-party payor coverage and reimbursement;
- our ability to manufacture MGTA-456 or any other product candidate in conformity with the U.S. Food and Drug Administration's requirements and to scale up manufacturing of our product candidates to commercial scale, if approved;
- our ability to successfully build a specialty sales force and commercial infrastructure;
- our ability to compete with companies currently producing or engaged in the clinical development of treatments for the disease indications that we pursue and treatment modalities that we develop;
- our reliance on third parties to conduct our clinical trials;
- our reliance on third-party contract manufacturers to manufacture and supply our product candidates for us;

- our ability to retain and recruit key personnel;
- our ability to obtain and maintain intellectual property protection for MGTA-456 or any other product candidates we may identify and pursue;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our needs for or ability to obtain additional financing;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act, or the JOBS Act;
- our financial performance; and
- developments and projections relating to our competitors or our industry.

Although we believe that the expectations reflected in these forward-looking statements are reasonable, these statements relate to our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from any future results, levels of activity, performance or achievements expressed or implied by these forward-looking statements. You are urged to carefully review the disclosures we make concerning these risks and other factors that may affect our business and operating results under "Item 1A. Risk Factors" in this Annual Report on Form 10-K, as well as our other reports filed with the Securities and Exchange Commission (the "SEC"). Any public statements or disclosures by us following this Annual Report on Form 10-K that modify or impact any of the forward-looking statements contained in this Annual Report on Form 10-K will be deemed to modify or supersede such statements in this Annual Report on Form 10-K. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this document. The Company does not intend, and undertakes no obligation, to update any forward-looking information to reflect events or circumstances after the date of this document or to reflect the occurrence of unanticipated events, unless required by law to do so.

PART I

Except where the context otherwise requires or where otherwise indicated, the terms "Magenta," "we," "us," "our," "our company," "the company," and "our business" refer to Magenta Therapeutics, Inc. and its consolidated subsidiary.

ITEM 1. BUSINESS

Overview

We are a clinical-stage biotechnology company developing novel medicines to extend the curative power of stem cell transplant, gene therapy, genome editing and cell therapy to more patients. We completed the initial public offering of our common stock in June 2018.

Transplant is a well-established and often curative medical procedure, and emerging data on stem cell gene therapy, which is stem cell transplant using gene-modified stem cells, suggest the potential for meaningful benefit with this newer form of transplant. Stem cell transplant and stem cell gene therapies use the same widely-adopted decades-old transplant process. As it exists today, stem cell transplant is a large market opportunity, and improvements to the current approaches could enable better stem cell transplant and extend stem cell transplant to more patients. The ability to treat patients with a stem cell transplant is limited by the challenges of obtaining sufficient cells to perform the procedure, the inherent morbidity and mortality of current methods used to prepare patients for transplant, and complications following transplant.

At Magenta, we believe we are uniquely positioned to overcome these challenges and to lead a new era in transplant medicine. Our portfolio of product candidates includes biologics, small molecules and a cell therapy designed to address deficiencies in existing approaches and extend the curative power of stem cell transplant, gene therapy, genome editing and cell therapy to more patients across many diseases. Currently, only a fraction of eligible patients with these diseases receive a transplant because the risks and challenges outweigh the potential for a cure. These include diseases where stem cell transplant is a standard of care (e.g., blood cancers such as acute myelogenous leukemia, myelodysplastic syndromes, multiple myeloma, and non-Hodgkin lymphoma), diseases where transplant is performed but limited in use (e.g., hemoglobinopathies such as sickle cell disease and beta-thalassemia), and autoimmune diseases. Emerging clinical data suggest that stem cell transplant may represent a breakthrough approach with curative potential for patients with severe autoimmune diseases. For example, recent results from multiple clinical trials show that patients with autoimmune diseases, including multiple sclerosis and scleroderma, can be cured with a transplant. However, based on our epidemiology analyses, currently only approximately 1 to 2% of eligible patients with multiple sclerosis or scleroderma in the United States and Europe receive a stem cell transplant.

To address the major unmet medical needs in the existing stem cell transplant process, we are developing a stem cell biology discovery platform and comprehensive portfolio of novel therapeutics. Our programs will improve stem cell dose (expansion), stem cell collection (mobilization), patient preparation for transplant (conditioning) and potential post-transplant complications to address key limitations of the stem cell transplant process to allow more patients to benefit. Within our expansion program, MGTA-456 is a cell therapy used with curative intent, and it has the potential to allow more patients to have a better chance for a successful stem cell transplant. We are currently studying it in patients with inherited metabolic disorders and patients with blood cancers. Within our mobilization program, MGTA-145 is focused on enabling physicians to more easily harvest a greater number of blood stem cells, known as hematopoietic stem cells or HSCs, from patients and donors to improve patient outcomes and scale the capacity of transplant and apheresis centers. Our targeted transplant conditioning programs, which prepare the patient for transplant, are designed to selectively remove stem and/or immune cells from a patient prior to transplant, and to be far less toxic than the decades-old radiation and chemotherapy-based approaches which are still the only available options. Our post-transplant complications program is designed to target the donor immune cells within the patient that cause Graft vs. Host Disease, or GvHD, which can be a fatal complication of transplant.

We intend to become a fully integrated discovery, development and commercial company in the field of transplant medicine. We believe that our product portfolio will offer significant commercial synergies. We are developing our products so that they can each be used individually or in combination with each other. As a result, our portfolio could be utilized in a manner tailored to the patient's disease, such that a patient may receive more than one Magenta therapy as part of their individual transplant journey.

Our goal is to have three clinical programs moving forward in multiple indications by 2020, with a rapidly advancing portfolio and sustainable platform.

Background on Stem Cell Transplant

Bone marrow is the tissue inside the bones where HSCs are located. HSCs produce all of the cells in the blood and immune systems, including: T cells and B cells to fight infections; red blood cells to carry oxygen; and platelets to control bleeding. The bone marrow is highly active, and gives rise to billions of new cells every day. However, abnormal functioning of the system can lead to serious and sometimes fatal blood and immune diseases. The aim of stem cell transplant, also called HSC transplant or bone marrow transplant, is to replace the diseased blood and immune cells with new stem cells that will produce new blood and immune cells, thereby effectively resetting the blood and immune systems to a healthy state. Stem cell transplant is a well-established procedure with curative intent, rooted in decades of clinical experience.

Starting from the first procedure in 1956, more than 1 million patients with blood cancers and genetic diseases worldwide have undergone a stem cell transplant, and currently approximately 65,000 patients undergo the procedure annually. Stem cell transplant serves as the standard of care in many blood cancers, such as leukemias, lymphomas and multiple myeloma; and for rare genetic diseases such as Hurler syndrome and is the only curative option for sickle cell disease.

However, stem cell transplant is a complex procedure that carries significant risks, including serious complications such as infection, secondary cancers and even patient death. A majority of patients who could significantly benefit from or be cured by a transplant do not receive one primarily because of the toxicity and risks associated with the procedure. For example, primarily because of these risks, approximately two out of three eligible patients suffering from acute myeloid leukemia do not receive a transplant despite it being the standard of care. For debilitating but non-life-threatening diseases, such as sickle cell disease and multiple sclerosis, even fewer patients are offered and receive a transplant because the risks of the current transplant procedure often outweigh the potential benefits. There is a clear need to improve the safety and efficacy of transplant so that we can refocus the patient and physician conversation and bring the curative power of transplant to more patients.

Our Strategy

Our mission and our culture are centered around the goal of enabling more patients with severe or life-threatening diseases to have access to the transformative benefit of a stem cell transplant. We intend to provide physicians with a tailored, multi-product treatment regimen based on the disease setting and the individual needs of each patient. Our strategic priorities are as follows:

***Bring the curative power of stem cell transplant, gene therapy, genome editing and cell therapy to all patients who can benefit by advancing an integrated product portfolio*:** We believe we are the only company that is committed to addressing the major limitations and challenges of stem cell transplant and transplant-based therapies, such as stem cell gene therapy, to revolutionize this entire field of medicine. Our product engine is generating a comprehensive portfolio of therapies to optimize the stem cell transplant process. Our initial focus is on inherited genetic diseases, blood cancers and autoimmune diseases, and we also plan to address other diseases for which transplant could represent a one-time, curative treatment.

***Build on our deep expertise in stem cell biology to lead a new era in transplant medicine*:** We have assembled a group of world leaders and pioneers in the fields of stem cell biology, biotherapeutics and transplant medicine. With this team, we are converting recent scientific breakthroughs into a product engine for stem cell transplant therapies.

***Create a fully integrated patient-focused biotechnology company*:** We are building a fully integrated biotechnology company with end-to-end capabilities in research, development and commercialization, and we believe the broad and synergistic nature of our portfolio will allow us to address many of the significant limitations of stem cell transplant and transplant-based therapies.

***Commercialize our therapeutics to bring tailored transplant solutions to physicians and patients*:** We are developing our portfolio of products so that they can be used individually or in combination with each other, such that a patient may receive more than one Magenta therapy as part of their individual patient journey. Our commercial model centers around hospital-based prescribers, and this is consistent across all of the products in our portfolio. Stem cell transplants are performed in a few hundred accredited medical centers in the U.S. and Europe, with more than half of the U.S. procedures performed at the top 20% of transplant centers. We believe the synergies among our programs and the well-defined structure of the current stem cell transplant provider network will allow us to commercialize all of our own therapeutics in the U.S. and Europe through a focused, targeted commercial organization.

***Leverage our two most advanced product candidates, MGTA-456 and MGTA-145, as clinical catalysts and commercial beachheads for our portfolio*:** In addition to the potential to bring meaningful clinical benefit to patients, MGTA-456 and MGTA-145 provide strategic value to Magenta by allowing us to accelerate the build-out of our clinical development infrastructure and footprint and to establish key customer relationships that will be important for future commercialization of all of our products.

***Continue to integrate our innovative collaboration with Be The Match with our science, medicine and business approaches*:** Be The Match is a leading patient-focused stem cell transplant organization in the U.S. Because of our shared patient focus, we and Be The Match have established a broad, first-of-its-kind collaboration. This collaboration positions us as a partner with high-priority access to many services that will continue to enable us to establish relationships across transplant centers and with key transplant physicians. Through our partnership, we access clinical strategy support; clinical development operational support, including a unique cell supply platform that is very well established at Be The Match and which will also enable our commercialization efforts across several programs. We also have access to the Be The Match payer and policy group to inform and support our pricing and reimbursement plans across the portfolio.

***Strategically collaborate to realize the full potential of our portfolio*:** We currently retain 100% of the commercial rights for our products, including all uses of MGTA-456 other than gene-modified stem cells. We will evaluate additional collaborations to:

- maximize the patient impact of our portfolio by partnering to enable gene and cell therapies, including stem cell-based gene therapies, genome editing and CAR T therapy;
- build relationships with partners to access complementary expertise and capabilities to bring our therapies as quickly as possible to all patients who can benefit; and
- opportunistically bring in preclinical or clinical assets that fit with our integrated portfolio.

Stem Cell Transplant: The Process and Challenges

A stem cell transplant procedure utilizes a number of integrated steps, which we highlight below: stem cell sourcing and collection, patient conditioning and stem cell infusion and engraftment. All transplants are categorized as either autologous or allogeneic, depending on the source of cells for the transplant. In an

autologous transplant, used for conditions such as multiple myeloma, non-Hodgkin lymphoma and autoimmune diseases, the patient's own stem cells are used. In the case of stem cell gene therapy and genome editing, once the cells are collected from the patient, they are then modified to either insert a functioning gene or correct a defective gene.

In an allogeneic transplant, used for conditions such as acute leukemia and genetic diseases, patients receive cells from a stem cell donor or umbilical cord blood.

Step 1: Stem Cell Sourcing and Collection

Once the patient and physician agree that stem cell transplant is the best treatment option, first the source of stem cells must be identified and then the cells are collected. There are three sources of stem cells for transplant:

- extraction from the bone marrow, which requires a procedure performed under general anesthesia where cells are withdrawn directly from the bone marrow with needle aspirates;
- mobilization into the peripheral blood, which currently requires several days of injections of a drug or combination of drugs to mobilize the cells, or move them from the bone marrow into the bloodstream, where they are then collected through a process called apheresis; and
- harvesting from umbilical cord blood units, which are stored in cord blood banks.

Challenges

Finding a donor: For patients undergoing autologous transplant, the patient's own cells are used for the transplant. For patients requiring an allogeneic transplant, the preferred source of stem cells is a donor from their family who has a well-matched immune system. For patients without a matched, related donor, the second option is a matched, unrelated donor identified through the bone marrow donor registry. Although there are nearly 35 million registered bone marrow donors worldwide, nearly half of all patients are unable to find a matched donor. Of the matched donors identified for patients, almost half decline to donate, which reduces the chance for finding a well-matched donor for the patient.

Stem cell collection: It is critical that physicians obtain enough stem cells for the transplant or gene therapy, whether from a patient or a donor, as higher cell doses are closely correlated to better patient outcomes. In some cases, multiple invasive bone marrow harvests are needed to obtain an adequate number of stem cells. The autologous transplant field has moved away from bone marrow harvest to stem cell mobilization in recent years due to the difficulty of the harvest procedure for both patients and physicians. In the case of stem cell mobilization, current treatments involve repeated injections over the course of several days and are often associated with bone pain, nausea, headache, and fatigue. Another challenge associated with current mobilization approaches is the difficulty of predicting whether mobilization will be successful, especially in heavily treated blood cancer patients. In fact, most patients require multiple days of apheresis and some patients require more than one mobilization procedure to obtain a sufficient number of cells for transplant. Additionally, mobilization failure rates for autologous transplants are as high as 40%.

For patients without a matched donor, other options include mismatched donors, who can either be unrelated or related; however, transplant outcomes are not optimal with these donor types. For patients without a matched, related donor, umbilical cord blood also provides a potential source of stem cells that is readily available. However, despite the utility of this approach, there are significant challenges with umbilical cord blood transplant. Even when patients find matched cord blood units, transplants are severely limited because there are often too few stem cells in the cord blood unit. Although more than 712,000 units are available in the worldwide cord blood inventory, current cell dose requirements mean that fewer than approximately 4% of these units contain enough stem cells for use in adult patients. Because so few units are suitable for use in adult patients, the patient is less likely to find a closely matched cord blood unit, leading to less than optimal transplant outcomes.

Step 2: Conditioning

Once sufficient cells have been obtained, the patient is then conditioned for transplant using systemic, toxic chemotherapy and/or radiation. Depending on the disease and type of transplant, the conditioning treatment is intended to remove or deplete:

- existing stem cells in the bone marrow to provide space for the incoming stem cells;
- immune cells (T cells and B cells) to prevent rejection of the incoming cells or remove disease-causing autoimmune cells; and/or
- cancer cells to prevent disease relapse in patients with blood cancers.

Challenges

Conditioning toxicity: Stem cell transplant and gene therapy conditioning is very burdensome and risky for both pediatric and adult patients. Conditioning treatments today are typically non-targeted and involve systemic, toxic chemotherapy and/or radiation. Most of these genotoxic chemotherapy agents, including derivatives of mustard gas, were discovered more than 50 years ago and were never intended for stem cell transplant conditioning. The current treatments eradicate the stem and immune cells and diseased cells but also indiscriminately damage DNA and kill normal, healthy cells in the body, which can lead to severe infections, organ failure, infertility, secondary cancers and even death. Nearly all transplant patients experience complications as a result of current conditioning treatments, and conditioning toxicity is responsible for up to 35% of mortality following allogeneic transplants. The severe immediate and long-term side effects and mortality risk of conditioning are among the major barriers preventing stem cell transplants from being performed more widely in other diseases where they may be curative. Efforts to reduce chemotherapy doses have met with some success, but the most commonly used conditioning regimens all involve the use of genotoxic agents.

Step 3: Stem Cell Infusion and Engraftment

Once conditioning is complete, the stem cells are infused into the patient via the bloodstream. The cells travel to the bone marrow and engraft there, meaning they lodge in the bone marrow and begin to make new blood and immune cells. Once the bone marrow has made enough blood cells – particularly white blood cells to fight infection – which typically takes several weeks, the patient can be discharged from the hospital. There are instances where the stem cells do not engraft or are rejected by the patient's body, leading to prolonged hospitalization and the need for an additional transplant. Outcomes of additional transplants are typically very poor.

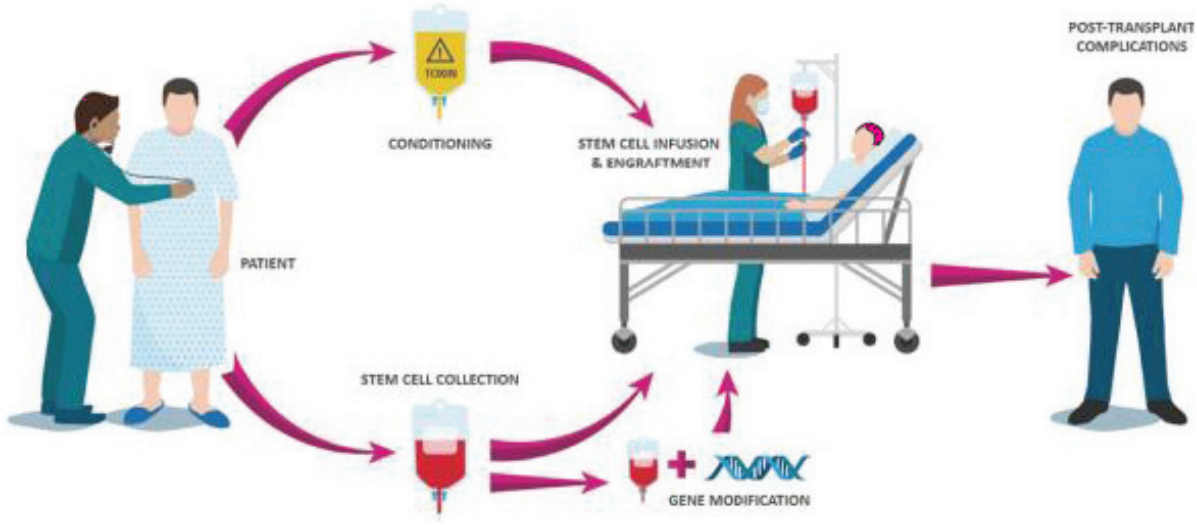
Challenges

Transplant rejection: A major complication of stem cell transplant is when the infused stem cells are rejected by the patient's body. Rejection is a particular problem where stem cell doses are low, for example with cord blood transplants.

Delayed engraftment: Depending on the dose of stem cells infused, engraftment can take between two to six weeks. During this period, patients are required to be in specialized isolation rooms at the hospital and are susceptible to infections.

Graft vs. Host Disease: GvHD is a reaction that commonly develops after an allogeneic stem cell transplant and is a result of the donor immune cells recognizing the patient's cells as foreign and attacking them. Acute GvHD typically occurs within weeks of a patient receiving a stem cell transplant and can severely damage the skin, liver, and gastrointestinal system. GvHD accounts for approximately 10% of deaths following allogeneic transplant.

Transplant Patient Journey



Our Solutions

We are applying our expertise in stem cell biology and biotherapeutics discovery to bring innovative, modern medicines to the transplant field through our programs, specifically designed to address each of the key challenges of the stem cell transplant journey for patients:

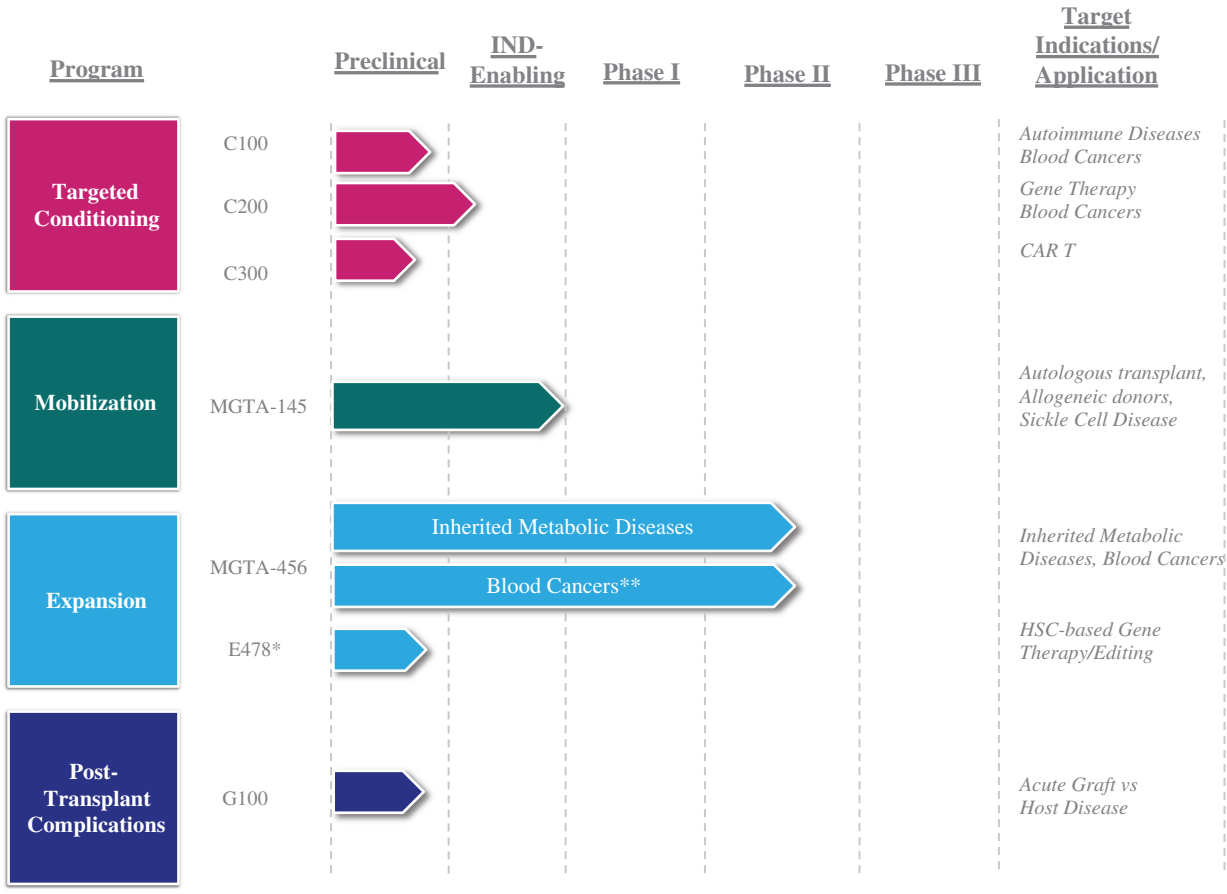
- ***Conditioning (targeted conditioning programs)***: Our targeted transplant conditioning programs are designed to selectively eliminate stem and/or immune cells from a patient prior to transplant or gene therapy, and to be far less toxic than the current radiation and chemotherapy-based treatments. These programs focus on developing targeted products that deplete specific cell types, with an approach that is tailored to the patient's disease and transplant requirements.
- ***Stem cell mobilization (mobilization program)***: Our stem cell mobilization program is focused on enabling physicians to more easily collect larger numbers of high-quality HSCs from patients and donors to yield higher cell doses and better outcomes for transplant as well as gene therapy.
- ***Stem cell source and engraftment challenges (expansion programs)***: Our stem cell expansion programs are focused on generating higher cell doses, which have been shown to improve the speed and success of engraftment in stem cell transplant and improve disease outcomes. Our AHR antagonist, E478, uses the same mechanism used to manufacture MGTA-456 to expand gene-modified HSCs for gene therapy and genome editing.
- ***GvHD (post-transplant complications program)*:** Our post-transplant program is designed to target the donor immune cells within the patient that cause GvHD, potentially reducing the occurrence of GvHD without sacrificing the benefits of an allogeneic transplant, broadening access to the curative potential of transplant.

Our Current Programs

We are developing a pipeline of small molecules; biologics, including antibody drug conjugates; and a cell therapy, which we believe can meaningfully improve and expand curative stem cell transplant options for many more patients with autoimmune diseases, blood cancers and genetic diseases. Our portfolio of novel medicines for transplant has the potential to allow more patients with debilitating or life-threatening diseases to access a one-time, transformative stem cell transplant with better outcomes, less toxicity risk and less mortality risk. We

are developing our product candidates so that they can each be used individually or in combination with each other, such that a patient may receive more than one Magenta therapy as part of their individual transplant journey. In addition to our first set of clinical and preclinical product candidates, we are in the process of identifying several other potential candidates from our discovery programs. Our goal is to have three distinct clinical programs in multiple indications by 2020 with a series of data read-outs.

Our Pipeline



* To be developed in partnership for E478-expanded gene therapies
** Investigator-initiated study

Our IND-Enabling and Clinical-Stage Product Candidates

C100, C200, C300: targeted antibody-drug conjugates for conditioning

We are developing a suite of novel antibody-drug conjugates, or ADCs, for transplant conditioning, a step in the transplant process that is still dominated by the use of systemic chemotherapy agents and radiation. We are seeking to replace these non-targeted toxic conditioning agents with targeted ADCs. These drugs are designed for transplant and specifically deplete only the cell types required to be eliminated in order to perform a successful transplant. Certain ADCs are currently used to treat cancer by directing a toxin to specific cells using antibodies. Our programs are adapting this clinically validated modality for conditioning patients for stem cell transplant.

All of our conditioning programs share an ADC platform but differ in the targeted cell types. The C100 program targets HSCs, immune cells, and disease-causing cells, the C200 program targets HSCs and disease-

causing cells and the C300 program targets only immune cells. This is achieved by tuning the antibodies to specific cellular markers or receptors that are expressed on the particular cell types.

	C100	C200	C300
Lead target	CD45	CD117	Undisclosed
Cells removed	Stem and immune cells Disease-causing cells	Stem cells Disease-causing cells	Immune cells
Diseases	Autoimmune diseases Blood cancers	Gene therapy Blood cancers	CAR T

The first conditioning program in our portfolio is C100, under which we are developing ADCs that specifically deplete host HSCs and immune cells. Within our C100 program, our lead target is CD45, an important cell surface molecule broadly expressed throughout the hematopoietic and immune systems. We plan to develop C100 for use in patients with autoimmune diseases such as multiple sclerosis and scleroderma, and patients with leukemias and lymphomas. We plan to declare a development candidate in our C100 program in 2019, then move into investigational new drug, or IND, enabling studies in 2020.

Our most advanced conditioning program, C200, is designed to specifically deplete HSCs and disease-causing cells. Our lead ADC product candidate targets CD117, also known as c-Kit, which is highly expressed on HSCs and leukemia cells, making it an ideal target for conditioning across broad sets of diseases. This includes blood cancers as well as hemoglobinopathies like sickle cell disease and beta-thalassemia, with potential applicability in both stem cell transplant and stem cell gene therapy. We have declared a development candidate in our C200 program and have moved into IND-enabling studies. We plan to file the IND, for this program in 2020.

Our third ADC-based conditioning program, C300, targets T cells, a type of immune cell. T cell depletion is currently performed with highly toxic, non-specific drugs which can lead to immune deficiency, infections and other complications including secondary autoimmune reactions. We are pursuing targets expressed on the surfaces of T cells with the goal of offering a safer and more optimized targeted conditioning approach through T cell depletion before CAR T therapy for blood cancers, prevention of stem cell rejection prior to allogeneic stem cell transplant or achievement of immune system reset before autologous stem cell transplant in patients with autoimmune diseases.

MGTA-145: CXCR2 agonist combined with plerixafor for HSC mobilization

Approximately 85% of stem cell transplants, or approximately 55,000 transplants per year in the U.S. and Europe, use mobilized peripheral blood from either donors or patients themselves as a source of stem cells. MGTA-145 is a novel stem cell mobilization product candidate that was developed based on our understanding of the physiological mechanisms that control stem cell mobilization. The goal of MGTA-145 is to achieve high levels of stem cell mobilization in patients and donors in the first-line setting and replace the current standard of care for mobilization, a drug known as granulocyte colony-stimulating factor, or G-CSF. G-CSF is a glycoprotein that mobilizes stem cells indirectly and comes with limitations that include a prolonged treatment period of up to one week of injections, lack of efficacy in some patients, and significant bone pain. MGTA-145 is a CXCR2 agonist protein that activates neutrophils to release proteases that cause the release of HSCs into the blood. This novel mechanism of action is synergistic with and complementary to that of plerixafor, another commonly used mobilization agent marketed by Sanofi. Plerixafor acts as a small molecule CXCR4 antagonist, blocking a pathway that otherwise plays an essential role in attracting and retaining HSCs in the bone marrow.

We plan to start the first clinical trial of MGTA-145 in the first half of 2019. This Phase 1 trial will study MGTA-145 plus plerixafor in healthy subjects. We plan to develop MGTA-145 for mobilization first in patients with multiple myeloma and non-Hodgkin lymphoma, and in healthy donors for allogeneic transplant. We also plan to study MGTA-145 in related and unrelated donors for allogeneic transplant; in patient populations where G-CSF can exacerbate the disease, such as autoimmune diseases; and patient populations where G-CSF is contra-indicated, such as sickle cell disease.

MGTA-456: cell therapy based on aryl hydrocarbon receptor (AHR) antagonist expanded stem cells

MGTA-456 is a novel proprietary allogeneic stem cell therapy that was developed based on our understanding of the mechanisms that control stem cell growth.

Larger stem cell doses and better matched transplants are both correlated with successful transplant outcomes, reduced risk of transplant failure, and faster time to engraftment and immune recovery. The goal of MGTA-456 is to allow more patients to have a successful transplant through higher doses of cells that are well matched to the patient.

We have initiated a Phase 2 study of MGTA-456 in patients with inherited metabolic disorders and intend to explore other debilitating diseases where we believe MGTA-456 could bring transformative benefit to patients. Although transplant can be curative in these diseases, between approximately 20 to 30% of patients with inherited metabolic diseases treated with transplant experience engraftment failure, resulting in severe complications, including death. We believe the high number of HSCs present in MGTA-456 should speed engraftment, reduce or eliminate engraftment failure and improve patient outcomes. Early results in patients with inherited metabolic disorders treated with MGTA-456 have shown promising signs of clinical benefit. If the results from this trial are favorable, we plan to initiate a Phase 3 trial in 2020.

MGTA-456 originally achieved human proof-of-concept in a Phase 1/2 study in patients with blood cancers. Data from the study showed that the median expansion of stem cells from the original cord blood unit was 330-fold, and all 36 patients treated with MGTA-456 in the study successfully achieved engraftment of these expanded stem cells. We are adding to the body of data for MGTA-456 in blood cancers through an investigator-initiated Phase 2 study in blood cancers at the University of Minnesota, which was initiated in December 2018. We anticipate that this study will triple the number of patients with blood cancers undergoing myeloablative conditioning who have been treated with MGTA-456, build experience with the cryopreserved formulation of MGTA-456 and help inform the design of further Company-sponsored studies.

We have also explored the possibility of studying MGTA-456 in patients with sickle cell disease. After careful review of comprehensive transplant outcomes data in sickle cell disease from the Center for International Blood and Marrow Transplant Research, or CIBMTR, we have concluded that the current transplant process will require optimization through targeted conditioning programs before MGTA-456 could deliver maximum clinical benefit to patients with sickle cell disease. We will continue our focus on advancing our potentially transformative conditioning and mobilization programs for patients with sickle cell disease.

On April 11, 2018, we received orphan drug designation from the FDA for MGTA-456 for the enhancement of cell engraftment in patients receiving stem cell transplant.

We are the sponsor of the IND for MGTA-456 in inherited metabolic diseases. The IND was originally filed by Novartis International Pharmaceutical Ltd, or Novartis, on November 6, 2015 and formally transferred to us on July 25, 2017. The IND is active and up to date with reporting requirements, such as annual reports. We obtained the rights to MGTA-456 through an April 2017 license agreement with Novartis granting us the sole worldwide rights to research, develop and commercialize certain AHR antagonist compounds specifically for the expansion of cord blood-derived non-gene-edited/-modified HSCs. See “Licenses and Collaborations” section.

E478: AHR antagonist for expansion of gene-modified stem cells

E478 is a novel and proprietary small molecule AHR antagonist that was developed to increase the number of gene-modified HSCs *ex vivo* for stem cell based-gene therapy. Gene therapy, or stem cell transplant with gene-modified or genome-edited cells, is a promising treatment approach for several diseases. However, this approach is significantly limited by the inability to generate a sufficient dose of gene-modified HSCs that retain the ability to engraft in patients as well as the cost and complexity of manufacturing viral vectors for gene modification of cells. These constraints could limit the commercial viability of this approach. E478 uses the same clinically validated mechanism as MGTA-456 to expand gene-modified HSCs. In addition to addressing cell dose limitations, the ability to expand long-term repopulating HSCs *ex vivo* has the potential to reduce manufacturing costs for these therapies by requiring less viral vector for gene modification of the stem cells. At the ASH annual meeting in December 2017, we presented data showing that culturing gene-modified human HSCs with E478 increased the number of gene-modified stem cells that retained engraftment ability in preclinical models. We are developing E478 specifically to partner with gene therapy and genome editing companies. E478 would be integrated into our potential partners' cell-based products leading to a newly defined cell therapy.

G100: ADC program for prevention of acute GvHD

We are developing a unique approach to preventing acute GvHD, a major complication and a leading cause of death in allogeneic transplantation. GvHD occurs when alloreactive T cells in the donor stem cell graft recognize the patient as foreign and attack their tissues. Current treatments for acute GvHD prevention include the prophylactic use of non-specific immune suppressive agents. The use of high doses of non-specific immune suppressive agents for GvHD treatment is correlated with an increased risk of infection and poor immune function, and despite the use of these powerful immune suppressive agents, approximately 50 to 80% of allogeneic transplant patients will experience acute GvHD. Our G100 program is designed to selectively eliminate only the components of the graft that cause acute GvHD, specifically the alloreactive T cells. This ADC therapy is intended to be dosed *in vivo* at the time of transplant. By specifically targeting the alloreactive T cells that arise shortly after transplant, this therapy should spare the remainder of the patient's immune system to allow immune recovery and protection from infections.

Background on Our Current Programs

	Unmet Needs	Magenta Program	Magenta Value Proposition
Patient Conditioning 	Genotoxic patient conditioning with significant side effects	CONDITIONING Antibody Drug Conjugates	Minimize transplant-related mortality and morbidities Reduce toxicity Reduce relapse rates
Stem Cell Source 	Low stem cell engraftment and poor outcomes Limited access to well matched grafts	EXPANSION Small Molecule Ex Vivo Expansion	Improve disease outcomes with high doses of well matched cells Faster time to engraftment and immune reconstitution
Stem Cell Collection 	Non-robust stem cell mobilization with side effects	MOBILIZATION Biologic Combo Drugs	Safely and predictably mobilize stem cells without severe side effects Same-day dosing and apheresis for patient and donor benefit and to maximize operations efficiency
Post-Transplant Complications 	High risk of Graft vs. Host disease	Post-Transplant Complications Novel Therapeutics	Prevent acute GvHD

Targeted Conditioning Programs

Unmet need

For patients undergoing stem cell transplant, the toxicity and mortality associated with current conditioning protocols are significant challenges and prevent more patients from benefitting from a life-saving and potentially curative transplant procedure. In many diseases, physicians have needed to use the most aggressive conditioning regimens to generate the best efficacy outcomes for transplant and transplant-based therapies, such as stem cell gene therapy.

Conditioning treatments today are typically non-targeted and involve high doses of radiation and/or toxic chemotherapy. These chemotherapy agents, including derivatives of mustard gas, were discovered more than 50 years ago and were never intended for stem cell transplant conditioning. The current treatments eradicate the stem and immune cells and diseased cells but also indiscriminately damage the DNA and kill normal, healthy cells in the body. These conditioning regimens can cause long-term lung injury and liver toxicity, serious infections, organ failure, infertility, secondary cancers and even death. Nearly all transplant patients experience complications as a result of current conditioning treatments, and conditioning toxicity is responsible for up to 35% of mortality following allogeneic transplants.

Emerging clinical data have shown that we are now at a stage where autoimmune disease can be cured with an autologous stem cell transplant, with recent data in multiple sclerosis and scleroderma. However, the toxicity of the required conditioning regimens has historically led many physicians to conclude that the risks of transplant in these patient populations far outweigh the benefits. A pair of comprehensive reviews published in 2017 in *Nature* journals summarized the clinical transplant results in multiple sclerosis and broader rheumatic autoimmune diseases. The main conclusions from this combined experience in more than 3,000 patients during the past 20 years were that the therapeutic benefit of stem cell transplant is significant across multiple studies in

severe autoimmune diseases: Relapsing remitting multiple sclerosis, or RRMS, (five Phase 2 trials) and systemic sclerosis (two Phase 2 trials and two randomized trials) have the most clinical evidence using stringent disease endpoints. When compared to the standard of care in RRMS, certain studies have shown that the proportion of patients with clinical benefit at two years appears to be double that of the next best treatment. Another study showed complete long-term suppression of all inflammatory activity in a cohort of patients with active and progressing multiple sclerosis who received a stem cell transplant, and a third study compared transplant to disease-modifying therapies and found that transplant resulted in prolonged time to disease progression. Given a stem cell transplant's ability to durably eliminate relapses and disease activity in multiple sclerosis patients, we believe a safer transplant procedure would be a viable option for those patients with highly active disease beyond what therapeutics can manage. However, currently only approximately 1 to 2% of eligible patients with multiple sclerosis and scleroderma in the U.S. and Europe receive a stem cell transplant primarily because the risk of the procedure outweighs the benefits of a potential cure. We believe we can significantly expand the number of autoimmune patients who can benefit from transplant.

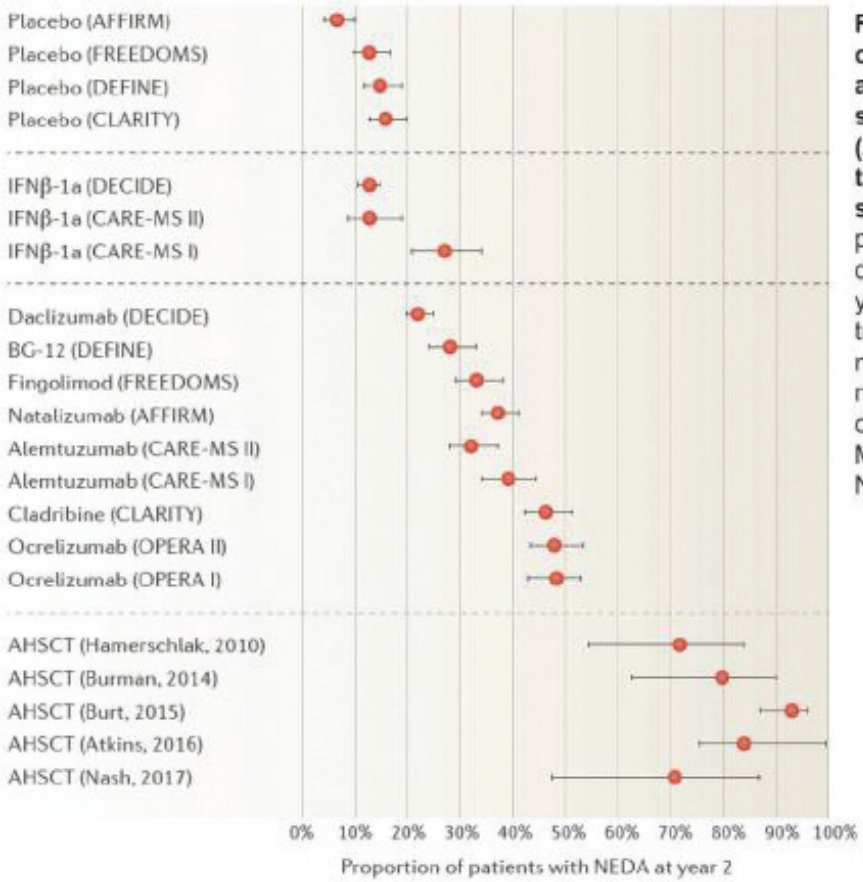
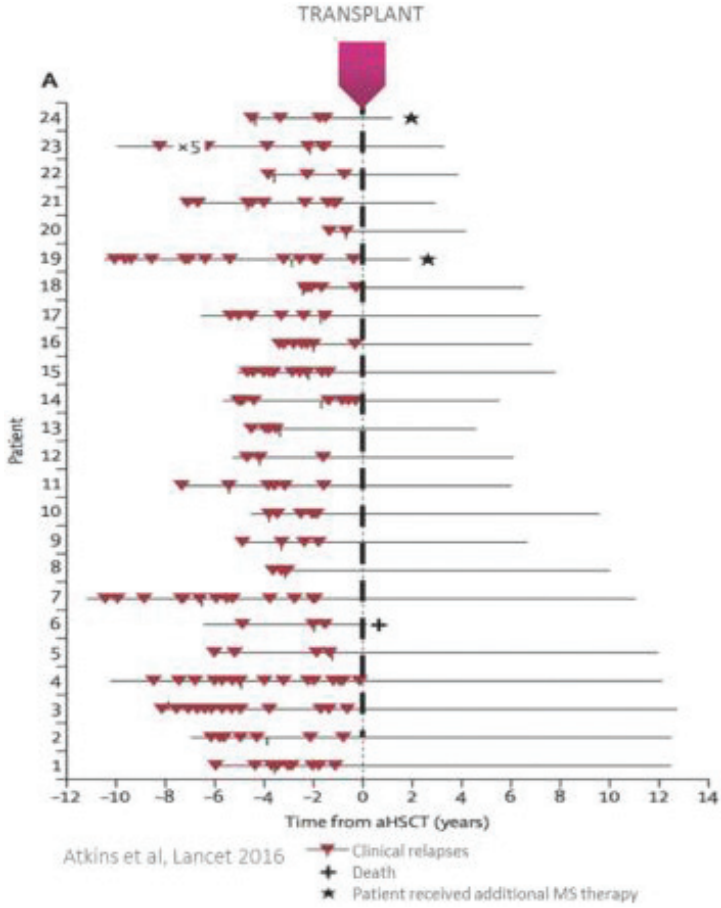


Figure 1: Durability of clinical benefit of autologous hematopoietic stem cell transplant (AHSCT) compared to other therapies for multiple sclerosis. The proportion of patients with no evidence of disease activity (NEDA) at 2 years during published clinical trials of AHSCT and disease-modifying drugs is shown by mean values with 95% confidence intervals (from Muraro et al, Nature Reviews Neurology, 2017).



Timeline of clinical relapses. Each line represents a patient treated in the study. The lines continue until the latest follow-up assessment, death (cross), or censoring because the patient received a conventional, experimental, or unproven treatment (star). The lines begin at diagnosis of multiple sclerosis, inverted triangles denote a clinical relapse.

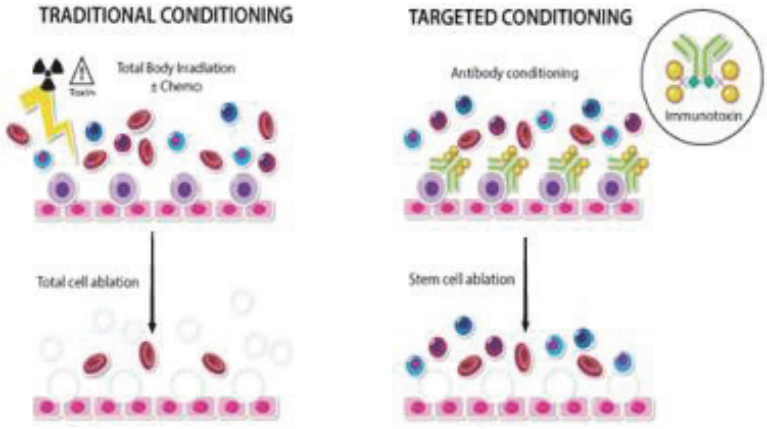
In January 2018, a paper was published in the New England Journal of Medicine demonstrating that hematopoietic stem cell transplant with myeloablative conditioning for patients with scleroderma resulted in long-term benefit, including improved event-free and overall survival; however, these benefits came with increased toxicity due to the non-targeted myeloablative conditioning used in the study.

Our solution

We are developing a suite of novel ADCs for transplant conditioning. We are seeking to replace the current non-targeted toxic conditioning agents with targeted ADCs designed for transplant that specifically deplete only the cell types required to be eliminated in order to perform a successful transplant. While ADCs are an established treatment for certain cancers, we believe this is the first time that ADC technology has been harnessed for transplant medicine. These programs have the potential to expand the curative power of transplant to every eligible patient, as well as beyond the eligible patient populations to patients who cannot tolerate current conditioning regimens. This includes patients with autoimmune diseases, such as multiple sclerosis, where the current risk-benefit of transplant is not considered favorable due to the toxicity of the existing conditioning regimens.

ADCs are a technology developed over the past 20 years where a monoclonal antibody specific for a cell surface protein is coupled to a drug. The ADC binds the receptor on the target cell, is internalized and degraded

to release the drug into the target cell. Coupling the drug to the antibody increases the specificity of drug delivery to the target cell, reducing systemic exposure and increasing the safety and efficacy compared to delivering the drug alone or the antibody without the drug attached. Today most ADCs are directed toward treating cancer cells expressing specific target receptors enriched on tumor cells. Our programs build on this clinically validated modality and adapt it for conditioning patients for stem cell transplant.



Targeted conditioning is more specific compared to traditional conditioning. Traditional conditioning is performed with total body irradiation and chemotherapy which eliminates all hematopoietic cells and nonspecifically damages other organs. Targeted conditioning with an antibody drug conjugate specifically eliminates the hematopoietic stem cells while sparing the immune system and avoiding systemic side effects.

In our development of ADCs for use in conditioning, we are optimizing for several key parameters:

- First, the antibody must specifically target a receptor that is expressed on the cells of interest, but not on other cell types.
- Second, to comply with typical stem cell transplant conditioning timelines, the antibody must have suitable efficacy to ensure that the ADC is able to kill the target cells rapidly, in days rather than weeks or months.
- Third, the antibody clearance needs to be accelerated so that it is eliminated by the time the transplanted cells are infused into the patient, typically within a week of starting conditioning. This requirement stems from the fact that the target receptor is expressed on cells present in the patient but also on the similar cell types in the transplanted cells.
- Finally, the linker-drug must be able to kill non-dividing cells as most HSC and immune cells are not actively dividing. The ADC linker must be chosen to minimize damage to non-target cells.

We are addressing each of these requirements through careful selection of the appropriate target receptor as well as antibody properties, including binding site, affinity, half-life and linker toxin.

We are developing three distinct programs for conditioning that specifically deplete the particular cells that need to be removed to make room for the incoming cells. These programs share a common platform but differ in the cell types targeted: The C100 program targets HSCs, immune cells and disease-causing cells, the C200

program targets HSCs and disease-causing cells and the C300 program targets only immune cells. The lead antigen targets for C100 and C200 and potential applications for each program are listed in the table below.

	C100	C200	C300
Lead target	CD45	CD117	Undisclosed
Cells removed	Stem and immune cells Disease-causing cells	Stem cells Disease-causing cells	Immune cells
Diseases	Autoimmune diseases Blood cancers	Gene therapy Blood cancers	CAR T

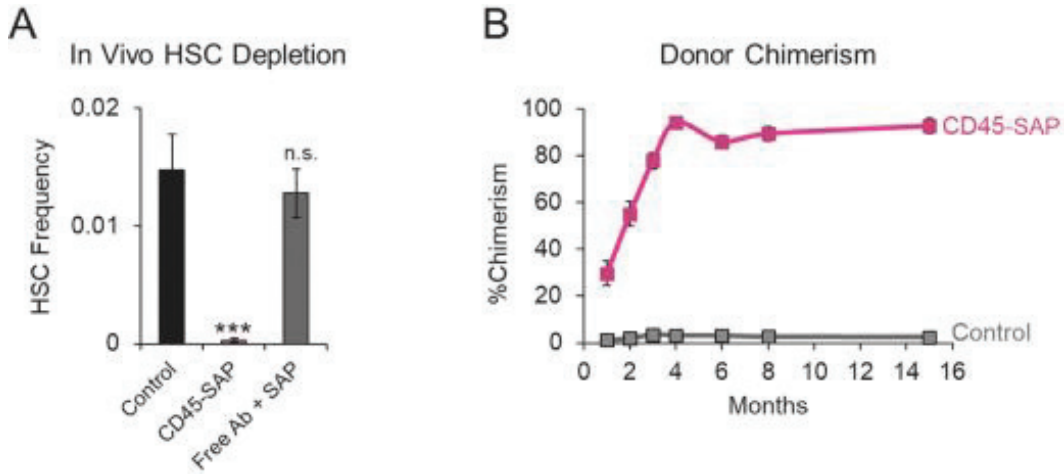
C100 program

The first conditioning program in our portfolio is C100, under which we are developing ADCs that specifically deplete host HSCs and immune cells. Within our C100 program, our lead target is CD45, an important cell surface molecule broadly expressed throughout the hematopoietic and immune system. We are currently in the lead identification stage for this program and intend to declare a development candidate in 2019, then move into IND-enabling studies in 2020.

C100-targeting HSC and immune cells

For many applications of HSC transplant, it is important to eliminate both immune cells and HSCs in the patient prior to transplant. This is especially important in the allogeneic setting where host immune cells can elicit an immune-mediated rejection of the incoming foreign stem cells. In addition, immune cell depletion is a key feature of the use of autologous transplant for the treatment of severe autoimmune disease. In this case, the goal is to eliminate the pathogenic auto-reactive immune cells that perpetuate the underlying autoimmune disease. Lastly, for patients with tumors expressing CD45, there may also be a direct anti-tumor effect providing additional therapeutic benefit. For these reasons, we are developing therapeutics that simultaneously target both HSCs and immune cells.

Pioneering work from Magenta founding scientists demonstrates that HSCs can be successfully targeted using a CD45-ADC to facilitate syngeneic transplant and allow for transplant-mediated cure of a mouse model of sickle cell disease. Importantly, CD45-ADC-treated mice show greatly reduced levels of organ damage and faster immune cell reconstitution and infection control compared to mice conditioned using radiation.

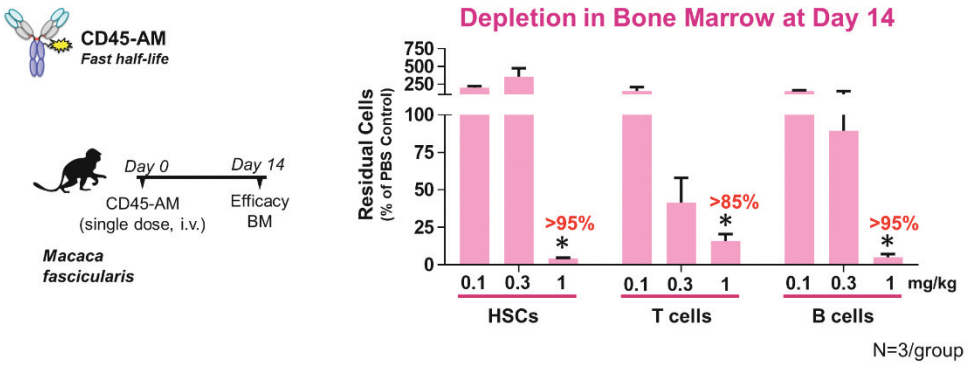


Source: Palchaudhuri et. al., Non-genotoxic conditioning for hematopoietic stem cell transplantation using a hematopoietic cell-specific internalizing immunotoxin. Nature (2016).

CD45–SAP has potent cell-depletion activity and enables efficient donor-cell engraftment. (A) CD45–SAP depletes HSCs in C57BL/6 mice whereas non-biotinylated CD45 antibody in the presence of streptavidin– SAP does not. Data represent mean ± s.d. (n = 5 mice/group, one of two independent experiments shown). HSCs were assessed by flow cytometry (Lin-cKit+Sca1+CD48-CD150+). **(B)** Long-term assessment of peripheral blood chimerism following CD45.2 GFP cell transplantation 8 days post CD45–SAP conditioning; all data points significant vs. control ($P < 0.05$). Data represent mean ± s.d. (n = 5 mice/group, assayed individually).

To translate these initial mouse studies into human therapeutics, we have deployed our biotherapeutics and stem cell biology platforms to discover and develop anti-human CD45 ADCs. These platforms include antibody discovery campaigns to identify high-affinity antibodies able to bind to human CD45 protein and cell lines expressing CD45. Conjugation of these candidate antibodies to select toxins and testing in functional killing assays in cell lines expressing CD45 and human HSCs growing *in vitro* is used to select lead antibodies for characterization *in vivo*.

We have developed CD45-amanitin ADC, an ADC using amanitin, an RNA polymerase II inhibitor capable of depleting cycling and non-cycling cells. In data presented at the Annual Meeting of the Transplant and Cellular Therapy, or TCT, in February 2019, we demonstrated that a single administration of CD45-amanitin ADC is capable of potent killing of CD34+CD90+ HSCs *in vitro*; depletion of human immune cells and CD34+ cells in the bone marrow of humanized NSG mice; and depletion of immune cells and CD34+ cells in the bone marrow of non-human primates.

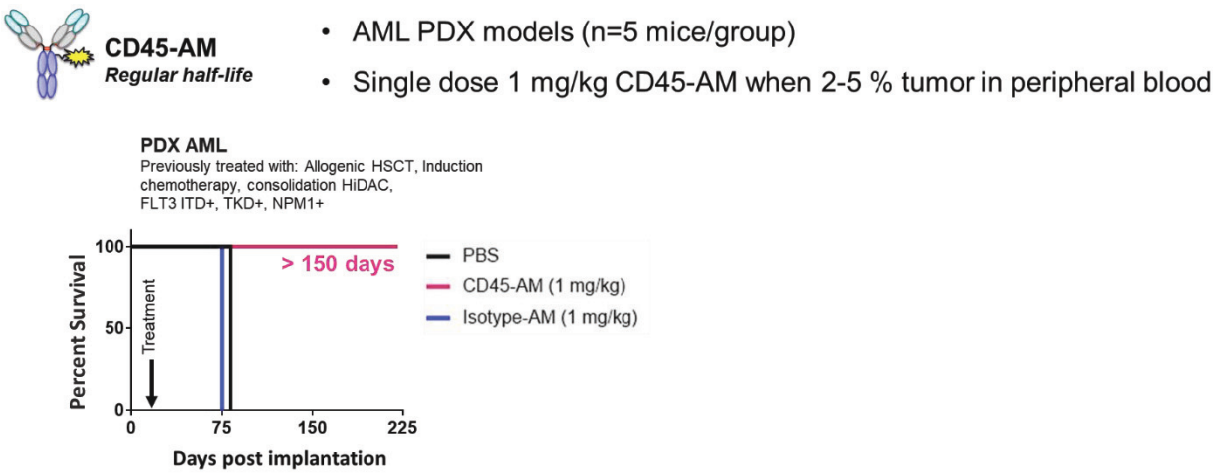


- Decrease in HSCs (CD34+ CD90+ CD45RA-) and immune cells in bone marrow at day 14
- No depletion observed with the isotype-amanitin conjugate or unconjugated antibody

Fast half-life anti-CD45-amanitin ADC effectively depletes HSCs and immune cells in bone marrow in non-human primates.

In additional data presented at the TCT meeting in 2019, the anti-CD45 amanitin ADC showed potent killing of human HSCs and human leukemia cell lines expressing CD45 *in vitro*. A single dose of CD45-amanitin ADC showed potent *in vivo* anti-leukemia activity in mice bearing established human leukemia cell lines. A single dose of the ADC also significantly improved the survival of mice engrafted with human leukemia cells

from acute myeloid leukemia patients, including leukemias that were resistant to multiple lines of therapy including previous allogeneic stem cell transplant.



Single Dose of Anti-CD45-Amanitin ADC Shows Potent Anti-Leukemia Activity in Patient-Derived Acute Myeloid Leukemia Models.

The CD45-amanitin ADC was well tolerated at the efficacious dose with no significant change in liver enzymes or other clinical chemistry parameters.

We anticipate nominating a development candidate for the C100 program in 2019, then beginning IND-enabling studies in 2020.

Clinical development plans

We believe C100 has the potential to increase the number of eligible patients who receive a transplant and expand the number of patients who are eligible. Non-genotoxic conditioning with a CD45-ADC may provide a novel approach for safer conditioning prior to stem cell transplant and greatly increase the number of patients eligible for a stem cell transplant. We believe a CD45-ADC may have applications in stem cell transplant for patients with autoimmune diseases and acute leukemias.

Multiple sclerosis affects approximately 600,000 to 700,000 patients in the U.S. and historically at least 650,000 patients in Europe. Approximately 15,000 new patients in the U.S. and 32,000 new patients in Europe are diagnosed annually. To assess eligibility in this population, we focused on the patients with active relapsing-remitting disease and relapsing secondary progressive multiple sclerosis patients who are not adequately treated by current therapies. This population represents approximately 45,000 multiple sclerosis patients who switch therapies each year in the U.S., and we believe that a greater population of patients switch therapies each year in Europe. Many of these patients switch because their current therapy does not adequately control disease activity that causes relapses. Given stem cell transplant's ability to durably eliminate relapses and disease activity in multiple sclerosis patients, we believe a safer transplant procedure would be a viable option for those patients with highly active disease beyond what therapeutics offer. Currently only approximately 1 to 2% of eligible multiple sclerosis patients in the U.S. and Europe receive a stem cell transplant because the risk of the process outweighs the benefits of a potential cure, and we believe we can significantly expand this number.

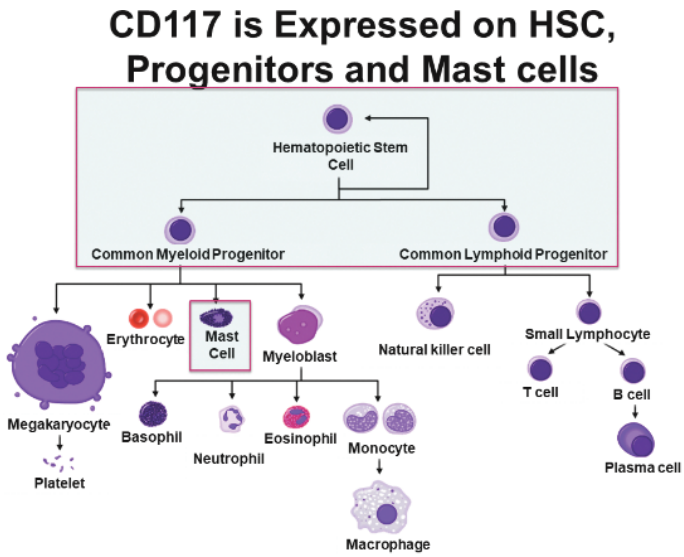
Scleroderma, a severe chronic connective tissue disease that is characterized by thickening of the skin and fibrosis in multiple organs, is diagnosed in approximately 6,600 patients in the U.S. and 10,900 patients in Europe annually. Approximately 35% (2,300 patients in U.S., 3,800 patients in Europe) of scleroderma patients suffer from the diffuse cutaneous form of the disease and are therefore eligible for stem cell transplant. Currently,

only approximately 1% or less of eligible scleroderma patients in the U.S. and Europe receive a stem cell transplant, though the recent inclusion of stem cell transplant in the European League Against Rheumatism, or EULAR, treatment guidelines for scleroderma may increase this number. With this increased acceptance of stem cell transplant as a treatment option for scleroderma and the opportunity for a safer transplant procedure through targeted conditioning, we believe transplant could become a new standard of care for patients with severe scleroderma, who have no other therapeutic options available.

There are approximately 72,000 cases of non-Hodgkin lymphoma diagnosed in the U.S. each year, of which approximately 6,500 are eligible for transplant; and in Europe there are approximately 95,000 new cases of non-Hodgkin lymphoma each year, with 8,500 eligible for transplant. Of these patients, approximately 60 to 80% receive a transplant. Approximately 47,000 patients in the U.S. and Europe are diagnosed with acute myelogenous leukemia every year. Of those patients, about 60% achieve remission with chemotherapy agents and are eligible for transplant. However, of these, only approximately 30 to 40% actually receive a transplant. This is largely due to the toxicity risk associated with current conditioning protocols. We believe our conditioning agents would allow more patients to achieve remission and become eligible for transplant.

C200 program

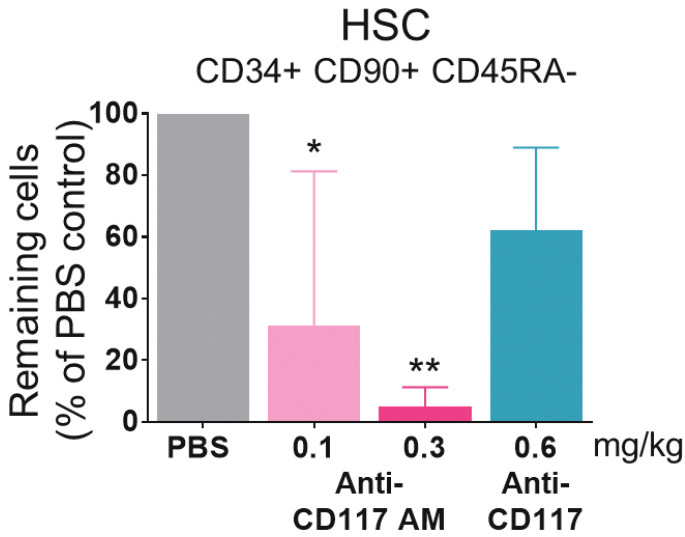
Our most advanced conditioning program, C200, is designed to specifically deplete HSCs and disease-causing cells. Our development candidate ADC targets CD117, also known as c-Kit, which is expressed on HSCs, making it an ideal target for conditioning across broad sets of diseases. This includes blood cancers, hemoglobinopathies (sickle cell disease and beta-thalassemia), and inherited metabolic diseases, with potential applicability in both stem cell transplant and HSC based gene therapy.



Based on: Nobili et.al., Long non-coding RNAs in normal and malignant hematopoiesis. Oncotarget (2016).

Initial experiments validated the concept of targeting CD117 using mouse models of HSC conditioning and transplant. In these studies, we found that a single dose of CD117-ADC was able to successfully deplete HSC in immunocompetent mice and allow successful transplant.

Based on these data, we commenced antibody discovery and optimization to develop an anti-human CD117-ADC. At the TCT conference in February 2019 we presented preclinical data showing that our CD117-ADC conjugated with amanitin potently depleted human and non-human primate HSCs and progenitors *in vivo*.

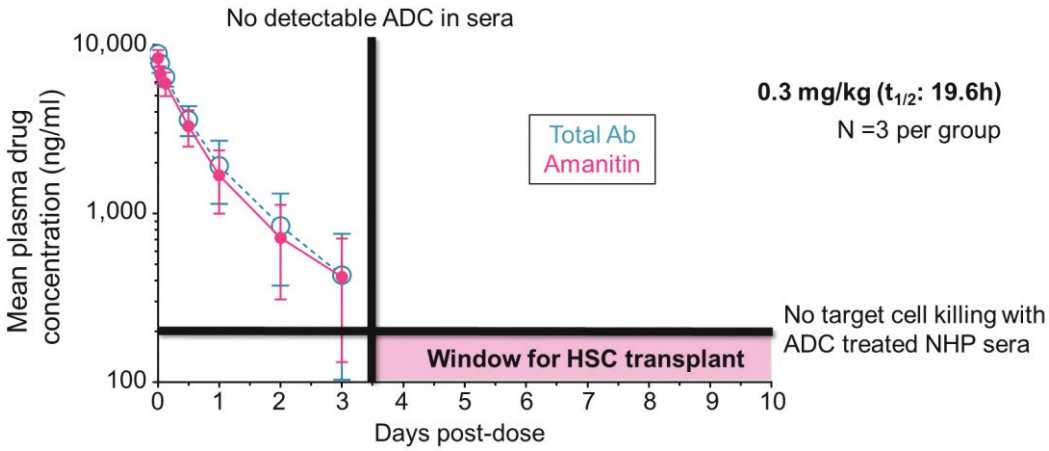


Engineered Anti-CD117-amanitin ADC Effectively Depletes HSC in Cynomolgus Monkeys. HSC numbers in the bone marrow seven days following a single dose of CD117-ADC or unconjugated CD117 antibody (CD117). The optimized CD117 amanitin ADC was able to eliminate 95% of HSCs in the bone marrow following a single dose of 0.3mg/kg. In contrast, treatment with an unconjugated CD117 antibody did not have a significant impact on HSCs.

In addition to dose-dependent depletion of HSCs in the bone marrow, the CD117 amanitin ADC led to transient and reversible reduction of reticulocytes in the blood, likely reflecting the fact that expression of CD117 is maintained throughout the early stages of red blood cell development.

The CD117 amanitin ADC was well tolerated at the efficacious dose. We observed no impact on lymphocyte levels, confirming the specificity of the CD117 amanitin ADC. The ADC led to low-level, transient, reversible elevation of liver transaminases that did not result in any adverse histopathological findings in either the liver or kidney at the effective dose.

Our CD117 amanitin ADC with engineered fast half-life showed potent stem cell depletion and rapid clearance in non-human primates, providing appropriate pharmacokinetics for patient preparation for stem cell transplant.

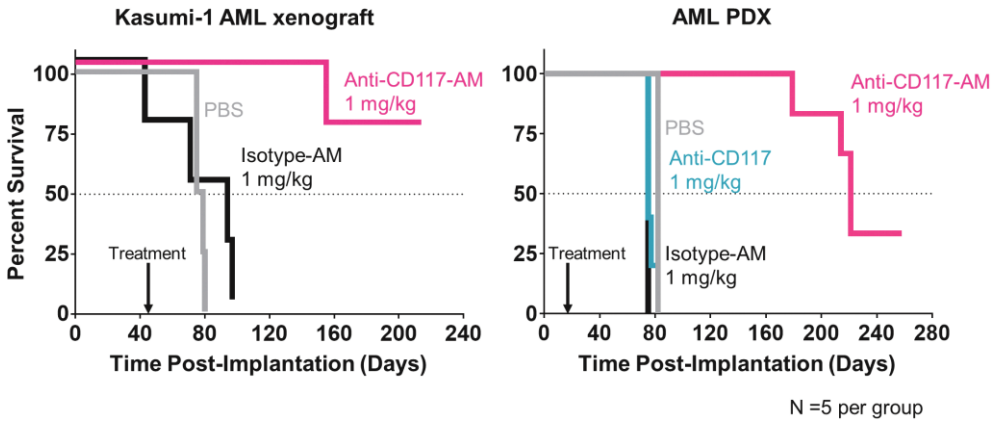


The timing of ADC-mediated depletion and clearance provides a window for transplant infusion.

Potent and selective depletion of stem cells with rapid clearance of a non-genotoxic ADC could provide a significant improvement over current approaches to patient preparation with an acceptable safety profile prior to stem cell transplant or gene therapy, broadening patient access to these potentially curative therapies.

Anti-tumor activity of CD117-ADC

In addition to its expressions on HSCs, CD117 is also frequently overexpressed on tumor cells in patients with acute myeloid leukemia and myelodysplastic syndromes. Thus, the CD117-ADC has the potential to reduce tumor burden in patients. In data presented at the TCT conference in February 2019, we showed that CD117-ADC was effective at killing human acute myeloid leukemia cells growing in vitro. To extend these data, we also assessed the ability of CD117-ADC to reduce tumor burden and confer a survival benefit in mice bearing a human acute myeloid leukemia cell line or patient-derived acute myeloid leukemias that were resistant to multiple lines of therapy, including previous allogeneic transplant. For these studies, mice were inoculated with a lethal dose of the acute myeloid leukemia and either left untreated or treated with CD117-ADC or unconjugated CD117 antibody. Tumor-bearing mice treated with a single dose of CD117-ADC showed significantly improved survival compared to mice left untreated or those treated with unconjugated CD117 antibody. These data suggest that CD117-ADC may have utility to treat leukemias through a direct cytotoxic effect on tumors.



CD117-ADC effectively depletes human leukemic cells in NSG mice. Schematic of *in vivo* leukemia model. The human acute myeloid leukemia cell line Kasumi-1 (left) or patient-derived acute myeloid leukemia cells (right) were injected into NSG mice to serve as a pharmacology model of established acute myeloid leukemia. A single injection of 0.3 mg/kg CD117 amanitin ADC or unconjugated anti-CD117 antibody or isotype amanitin occurred after tumors were established. Survival curves of mice after tumor injection and treatment are shown.

The targeted conditioning and potential for disease control offered by CD117-ADC may extend stem cell transplant to additional leukemia patients and improve outcomes. This is especially important as acute myeloid leukemia and myelodysplastic syndromes represent areas of high medical need. Current treatment for patients with high-risk acute myeloid leukemia or myelodysplastic syndromes involves chemotherapy to induce disease remission followed by allogeneic stem cell transplant. However, many patients are unable to achieve disease remission using current chemotherapy agents, rendering them ineligible for allogeneic transplant. Moreover, in the approximately 35% of eligible patients with acute myeloid leukemia and myelodysplastic syndrome that do receive a transplant, outcomes remain suboptimal with only 50% survival five years after transplant. In addition, many patients have co-morbidities such as increased age or decreased organ function that necessitate the use of a reduced intensity of conditioning which leads to higher rates of disease relapse. Because acute myeloid leukemia and myelodysplastic syndromes are increasingly seen in older patients, many patients are often deemed too frail to undergo a transplant, leaving them with limited treatment options. The anti-leukemia activity of CD117-ADC has the potential to reduce disease burden and improve outcomes for patients and allow relapsed/refractory patients to have access to a stem cell transplant.

We have declared a development candidate in our C200 program and have moved into IND-enabling studies. We plan to file the IND for this program in 2020.

Clinical development plans

Our goal is also to investigate the ability of CD117-ADC to condition patients prior to receiving HSC-based gene therapy. Gene therapy is a promising approach to treat a variety of non-malignant diseases, including inherited metabolic disorders, sickle cell disease and beta-thalassemia. However, the risks and toxicity associated with current chemotherapy-based conditioning approaches may limit the utility of this approach. Our targeted CD117-ADC has the potential to provide a safe and effective approach to conditioning gene therapy patients.

We believe CD117-ADC has the potential to increase the number of eligible patients who receive a transplant and expand the number of patients who are eligible.

Given the ability of CD117-ADC to deplete HSCs and leukemia cells shown in our preclinical data, we intend to pursue a development plan for patients with acute myeloid leukemia and myelodysplastic syndromes.

Approximately 47,000 patients in the U.S. and Europe are diagnosed with acute myeloid leukemia every year. Of those patients, about 60% achieve remission with chemotherapy agents and are eligible for transplant. However, of these, only approximately 30 to 40% actually receive a transplant. This is largely due to the toxicity risk associated with current conditioning protocols. We believe our conditioning agents would allow more patients to achieve remission and become eligible for transplant.

Approximately 34,300 patients in the U.S. and Europe are diagnosed with myelodysplastic syndromes every year. Of those patients, about 30% are eligible for transplant based on high risk disease. However, of these patients, only approximately 16 to 40% actually receive a transplant. This is largely due to the toxicity risk associated with current conditioning protocols. We believe our conditioning agents would allow more patients to achieve remission and become eligible for transplant.

C300 program

Our third ADC-based conditioning program, C300, targets T cells. T cell depletion is currently performed with highly toxic non-specific drugs, which can lead to immune deficiency, infections and other complications, including secondary autoimmune reactions. We are pursuing targets expressed on the surfaces of T cells with the goal of offering a safer and more optimized targeted conditioning approach through T cell depletion before CAR T therapy for blood cancers, prevention of stem cell rejection prior to allogeneic stem cell transplant or achievement of immune system reset before autologous stem cell transplant in autoimmune disease patients.

Stem cell mobilization program

Unmet need

Successful stem cell transplantation requires collection of HSCs in both sufficient number and quality to allow for robust engraftment, recovery of blood cells, and lifelong maintenance of the hematopoietic system. Higher cell doses are associated with better outcomes in stem cell transplant.

Current methods for stem cell collection include a bone marrow harvest, a procedure performed under general anesthesia, or mobilization of stem cells from the bone marrow to blood, where they are then collected through a process called apheresis. The autologous transplant field has moved away from bone marrow harvest in recent years, due to the difficulty of the procedure for both patients and physicians, and there is a need for better HSC mobilization agents for apheresis.

The predominant source of HSCs for adult transplant is mobilized peripheral blood that is acquired by apheresis, or collection of donor or patient blood after several days of injections of G-CSF. G-CSF mobilizes

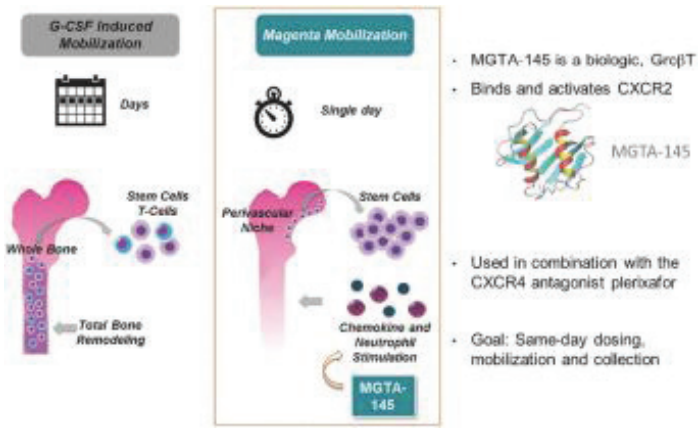
stem cells indirectly, requiring repeated daily injections, and is often associated with bone pain, nausea, headache, and fatigue. The multi-day regimen can take up to a week and side effects can be disruptive for both patients having their cells collected for autologous transplants and for healthy volunteers donating their cells for allogeneic transplants. It is difficult to predict whether mobilization will be successful, especially in heavily treated blood cancer patients. In fact, most patients require multiple apheresis sessions and some patients require more than one stem cell collection procedure to obtain a sufficient number of cells for transplant. Mobilization failure rates are as high as 35% in patients with multiple myeloma or non-Hodgkin lymphoma. For patients who are unable to mobilize sufficient numbers of stem cells with G-CSF, physicians are then required to retreat with G-CSF and add another drug, called plerixafor. Plerixafor acts as a small molecule CXCR4 antagonist, blocking a pathway that otherwise plays an essential role in attracting and retaining HSCs in the bone marrow. It is approved for use in combination with G-CSF for patients who fail to achieve sufficient mobilization of stem cells with G-CSF alone. It can mobilize stem cells as a single agent but not to sufficient levels to be used as a standalone agent. In addition, G-CSF is also contra-indicated in some patient populations, such as patients with sickle cell disease, and it can exacerbate autoimmune diseases.

Our solution

MGTA-145 is a novel first-line stem cell mobilization product candidate that was developed based on our understanding of the physiological mechanisms that control stem cell mobilization. The goal of MGTA-145 is to achieve high levels of stem cell mobilization in patients and donors in order to offer a predictable and reliable first-line mobilization agent that enables same-day apheresis, without the need to use G-CSF, the current standard of care. MGTA-145 is a CXCR2 agonist protein that activates neutrophils to release proteases that cause the release of HSCs into the blood. This novel mechanism of action is complementary to that of plerixafor. Preclinical data have shown that the synergistic combination of MGTA-145 and plerixafor leads to robust and rapid stem cell mobilization. Further, these mobilized stem cells outperform stem cells mobilized by G-CSF in mouse transplant models.

Mechanism of action

CXCR2 is a chemokine receptor expressed on the surface of neutrophils. Binding of MGTA-145 to the receptor results in neutrophil activation. Published data from Magenta founders and scientists show that a key event for mobilization of stem cells is the MGTA-145-mediated release of proteases from activated neutrophils, which together with the actions of the CXCR4 antagonist, plerixafor, results in the rapid release of HSCs from the bone marrow into the blood. Blocking CXCR4 using plerixafor and activating neutrophils with MGTA-145 together produce an effective and synergistic untethering and release of HSCs from bone marrow into the blood, resulting in rapid and robust mobilization of HSCs.

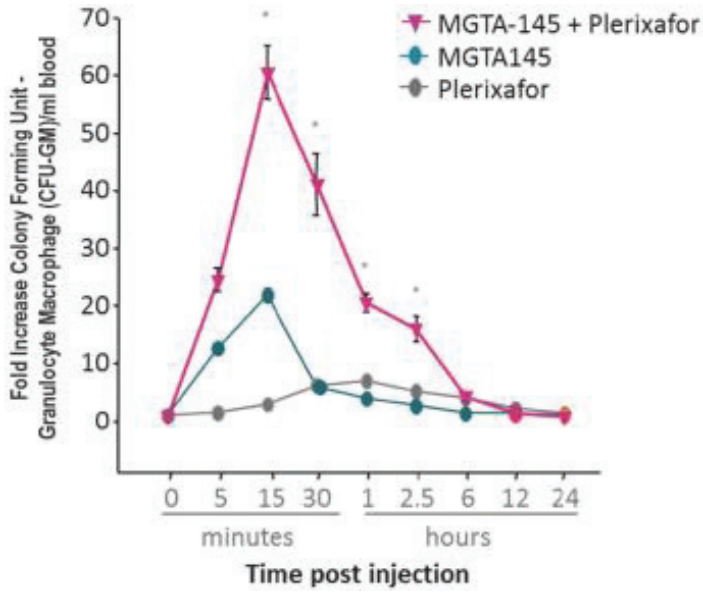


The goal of MGTA-145 is to enable single-day mobilization of high numbers of HSCs. Mobilized peripheral blood stem cells are currently the predominant source of HSCs for both autologous and allogeneic

transplantation. The most common clinical HSCs mobilization protocol is five days of G-CSF. This regimen requires daily injections, has been associated with bone pain and often results in unpredictably low yields. MGTA-145, in combination with plerixafor, provides a rapid mobilization method that only requires a single treatment and has robust and predictable kinetics. The stem cells mobilized with this combination contain a higher frequency of HSCs compared to those mobilized with G-CSF, which could significantly improve transplant outcomes over the current standard of care.

Preclinical development

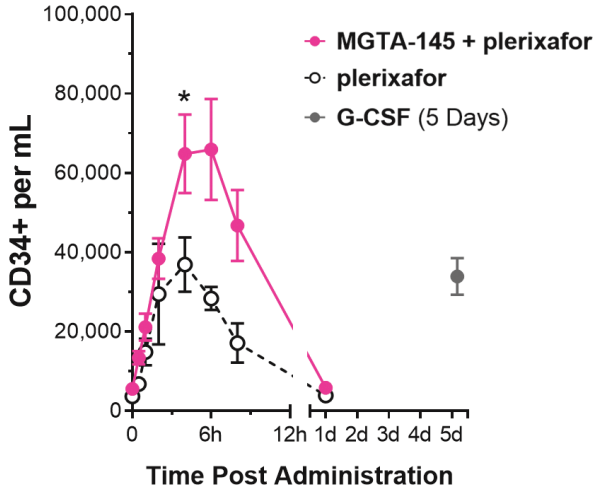
Research from Magenta scientific collaborators from Harvard and Massachusetts General Hospital published in *Cell* in December 2017 demonstrates that a single administration of MGTA-145, in combination with plerixafor, resulted in rapid mobilization of robust numbers of HSCs from the bone marrow and into the blood. The cells were then harvested for transplant in mouse models. Notably, HSCs mobilized with MGTA-145 and plerixafor and subsequently transplanted into mouse models resulted in faster engraftment and higher levels of donor cells, two important factors for the success of a transplant, compared to cells obtained with the current mobilization standard of care, G-CSF.



The combination of MGTA-145 and plerixafor rapidly mobilizes mouse HSCs. Blood was assessed at various time points after administration of plerixafor (5mg/kg), MGTA-145 (2.5mg/kg) or MGTA-145 plus plerixafor combination given simultaneously. Mean ± the standard error of the mean (SEM) from n = 4 BALB/c mice/group/time point. *p < 0.01 versus MGTA-145 or plerixafor ANOVA. (Ref: Hoggatt et al., 2018, Cell 172, 1–14).

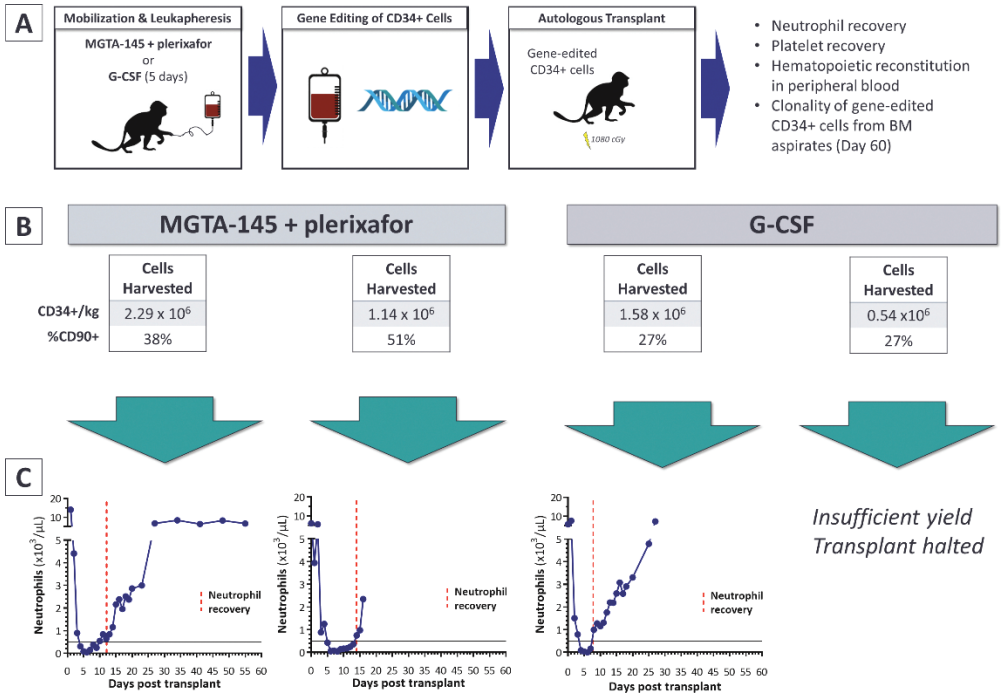
In studies in non-human primates, we showed that a single administration of MGTA-145, in combination with plerixafor, induced robust mobilization of a transplantable dose of CD34+CD90+CD45RA- HSCs within four hours of administration. CD34+CD90+CD45RA- cells are highly enriched for HSCs and are important for

long-term engraftment and hematopoietic reconstitution, both of which influence patient outcomes. These data were presented at the TCT conference in February 2019.



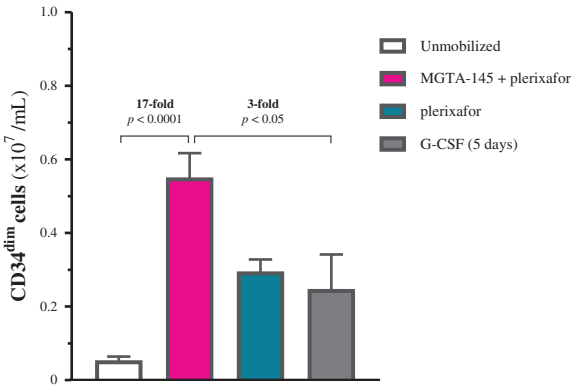
A single injection of MGTA-145 + plerixafor mobilizes higher numbers of CD34+ cells compared to a multi-day regimen of G-CSF in nonhuman primates. Rhesus macaques received a single injection of plerixafor (1 mg/kg SC) alone or MGTA-145 (450 µg/kg IV) + plerixafor or five daily injections of G-CSF (50 µg/kg SC). Treatment with MGTA-145 + plerixafor leads to robust CD34+ cell mobilization that peaks at 4-6 hours post dose administration. Data represent 3-13 animals per group and are expressed as mean +/- SEM. Statistical significance was determined by Student's t test. *p<0.05 for comparisons to plerixafor.

In further studies in non-human primates, MGTA-145, used in combination with plerixafor, rapidly mobilized robust numbers of stem cells compared to G-CSF. Two of two primates were successfully mobilized with this combination, while only one of two primates was successfully mobilized with G-CSF. The cells mobilized with the MGTA-145 and plerixafor combination successfully engrafted in a non-human primate model.

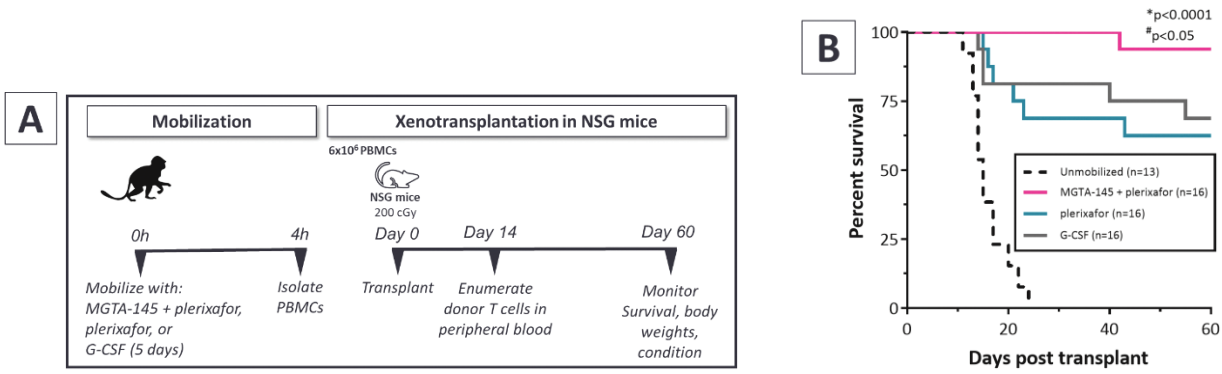


Autologous transplant of MGTA-145+ plerixafor-mobilized CD34+ cells leads to rapid neutrophil recovery in non-human primates. (A) Schematic for a non-human primate model of autologous transplantation of gene-edited CD34+ cells. Rhesus macaques were mobilized with a single injection of MGTA-145 + plerixafor or 5 daily injections of G-CSF and mobilized peripheral blood was collected by leukapheresis starting at 1-2 hours post dose administration. Mobilized CD34+ cells were isolated by positive selection and gene edited in a 48-hour culture prior to autologous transplantation back into rhesus macaques following TBI. **(B)** Apheresis yields and **(C)** time to neutrophil recovery are shown for 2 non-human primates transplanted with MGTA-145 + plerixafor-mobilized CD34+ cells compared to 1 animal transplanted with G-CSF-mobilized CD34+ cells. An additional non-human primate mobilized with G-CSF had insufficient CD34+ yield and was not transplanted due to safety concerns. All animals received daily injections of G-CSF (10 µg/kg) post transplant as supportive care to treat neutropenia until neutrophil counts passed 500/µL. The second non-human primate to receive MGTA-145 + plerixafor-mobilized CD34+ cells is at day 18 post-transplant, and the non-human primate that received G-CSF-mobilized CD34+ cells is at day 37 post-transplant.

Further, the combination of MGTA-145 and plerixafor mobilized a high number of a CD34dim population of monocytes with immunosuppressive properties that blocked GvHD, a significant challenge in allogeneic transplant, in a preclinical model.



MGTA-145 + plerixafor mobilized higher numbers of CD34^{dim} cells compared to G-CSF or plerixafor alone.



MGTA-145 + plerixafor mobilized cells capable of suppressing graft-versus-host-disease and extending survival. (A) Schematic for a mouse xeno-transplantation model to assess the development of acute GvHD following mPB transplantation is shown. **(B)** Percent survival of NSG mice transplanted with unmobilized versus MGTA-145 + plerixafor-, plerixafor- or G-CSF-mobilized PBMCs is shown. * for comparisons to unmobilized, # for comparisons to plerixafor. These data were presented at the TCT conference in February 2019.

Taken together, these data suggest that MGTA-145 with plerixafor offers a more robust, safer and more practical first-line therapy alternative to G-CSF for single-day mobilization and apheresis of large numbers of stem cells for use in both autologous and allogeneic transplant.

Clinical development plan

We plan to start the first clinical trial of MGTA-145 in the first half of 2019. This Phase 1 trial will study MGTA-145 plus plerixafor in healthy subjects. We plan to develop MGTA-145 for mobilization first in patients with multiple myeloma and non-Hodgkin lymphoma. We also plan to study MGTA-145 in related and unrelated donors for allogeneic transplant; in patient populations where G-CSF can exacerbate the disease, such as autoimmune diseases; and patient populations where G-CSF is contra-indicated, such as sickle cell disease.

We anticipate that the market opportunity for MGTA-145 will continue to increase as the number of transplants performed increases.

In the U.S. and Europe, approximately 85% of stem cell transplants performed each year use mobilized peripheral blood from either donors or patients themselves as a source of stem cells. The number of patients requiring mobilization may increase as safer transplant conditioning protocols, such as the ones we are developing, increase the number of patients eligible for transplant, including patients with autoimmune or sickle cell disease.

Stem cell expansion program: MGTA-456 cell therapy

Unmet Need

It is critical that patients receive a sufficiently high dose of HSCs in the transplant, as higher cell doses are closely correlated to better patient outcomes. In allogeneic transplant, up to half of the approximately 23,000 patients requiring donor cells will not find a suitable donor within their families or among the registered bone marrow donors. Long search times for unrelated donors and their frequent unavailability represent a major challenge, particularly for patients with diseases that can progress rapidly, such as certain inherited metabolic diseases and high-risk acute leukemias. Because of the urgency for these patients to undergo transplant, there is a need for new options, and the ability to quickly find a well-matched stem cell donor with sufficient cells remains one of the most significant hurdles in stem cell transplant.

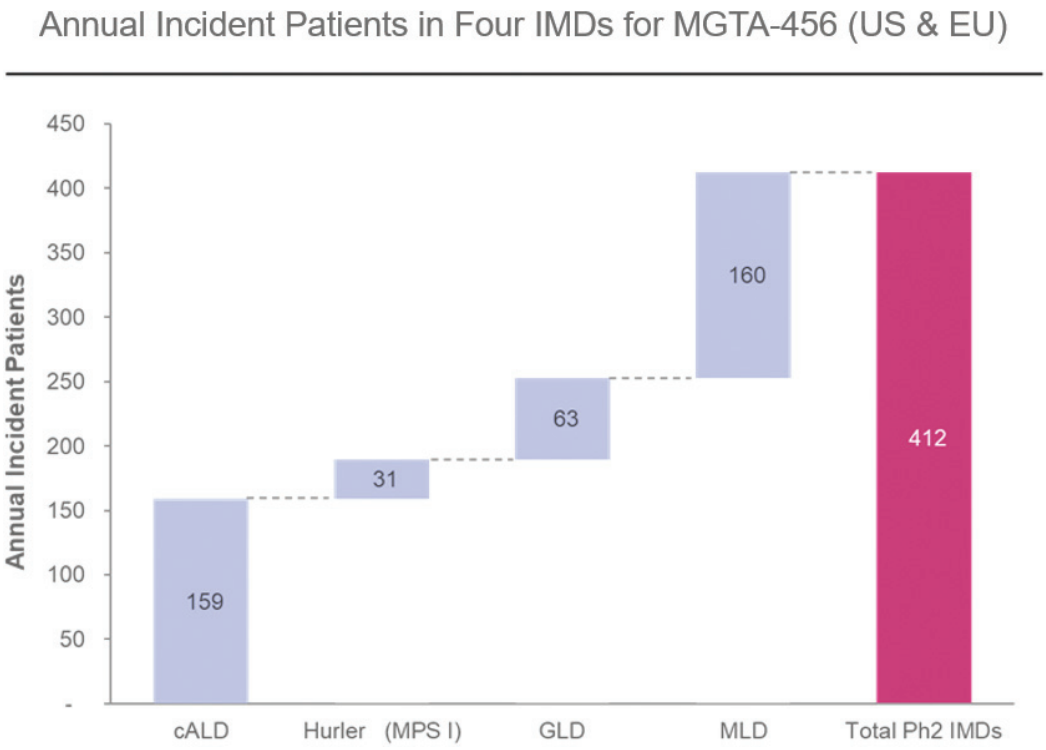
Our Solution

MGTA-456 is an allogeneic cell therapy that is readily available and is designed to provide patients with a higher dose of HSCs that are well matched to the patient.

Rare Genetic Diseases

We are currently studying MGTA-456 in a Phase 2 trial in patients with inherited metabolic disorders. These rare genetic diseases, which include Hurler syndrome, cerebral adrenoleukodystrophy, or cALD; metachromatic leukodystrophy, or MLD; and globoid cell leukodystrophy, or GLD; progress rapidly and are fatal if left untreated. Stem cell transplant is the only disease-modifying treatment option for Hurler syndrome, and the only treatment option for cALD, MLD and GLD. We believe the high number of HSCs present in MGTA-456

will speed engraftment, reduce or eliminate engraftment failure and improve patient outcomes, with the potential to deliver transformative and durable disease benefit to these patients.

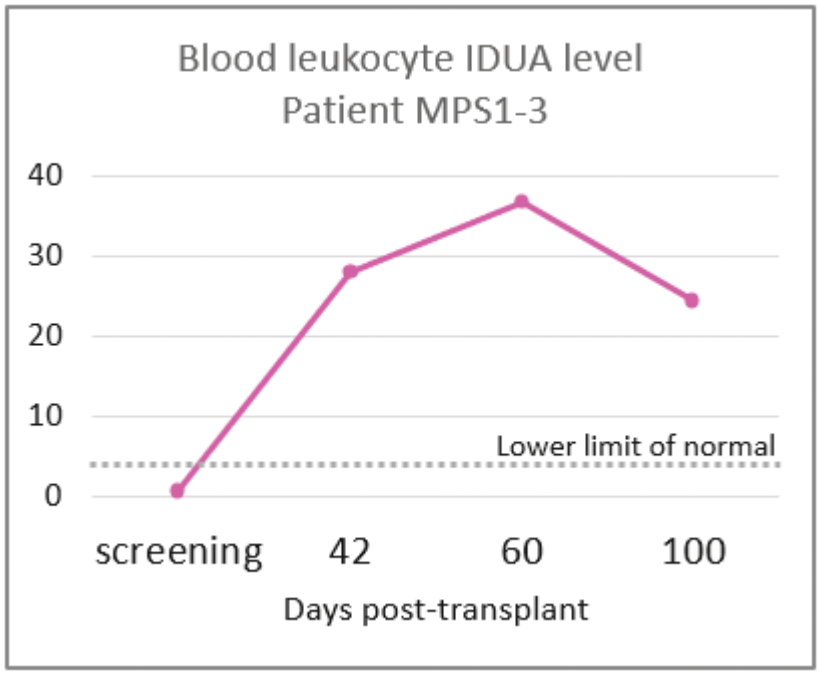


At the TCT conference in February 2019, we reported early data from the first five patients treated in this Phase 2 study. All five patients with inherited metabolic disorders treated with MGTA-456 as of the data cut-off met the primary endpoint of successful engraftment. Patients with Hurler syndrome showed an increase in corrected enzyme and decrease in disease-related metabolites, and patients with cALD showed resolution of brain inflammation on MRI as early as one month post-treatment. These early disease markers and brain imaging evidence, respectively, are correlated with positive long-term disease outcomes. Two treatment-related adverse events were observed: one grade 1 vomiting and one grade 3 nausea, both of which were transient.

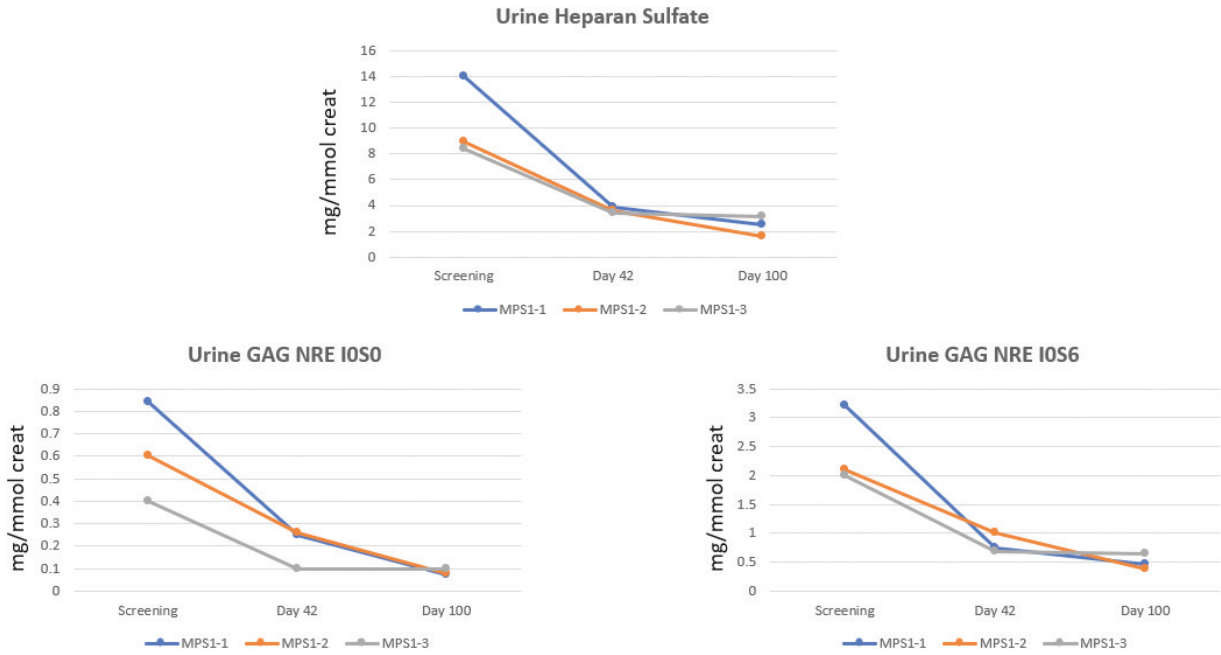
Day post-transplant	MPS1-1	MPS1-2	MPS1-3
Pre-transplant	Low	Low	Low
+42	Normal	Normal	Normal
+60	Normal	Normal	Normal
+100	Normal	QNS	Normal

(QNS: Quantity not sufficient)

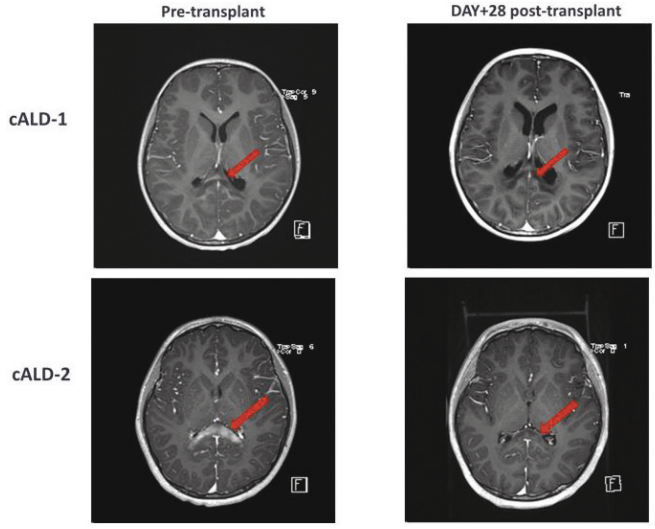
Normalization of enzyme activity in three patients with Hurler syndrome within 42 days of receiving MGTA-456.



Enzyme levels in a representative patient with Hurler syndrome.



Decrease in disease-related metabolites in three patients with Hurler syndrome.



Day post-transplant	Screening	+28	+60	+100	+180
Loes Score	3	3	3	3	3
Gadolinium enhancement	Y	N	N	N	N

Day post-transplant	Screening	+28	+60	+100
Loes Score	2	2	2	2
Gadolinium enhancement	Y	N	N	N

Resolution of gadolinium enhancement, a marker of brain inflammation, in two patients with cALD.

Two patients less than two years of age with Hurler syndrome treated with MGTA-456 in the Magenta study developed autoimmune cytopenia. Autoimmune cytopenia was also observed in one of the two patients with cerebral adrenoleukodystrophy in the study. This patient was successfully treated for the cytopenia and continued to show evidence of cALD disease benefit by MRI imaging as shown above.

These cytopenias were determined to be unrelated to MGTA-456. Autoimmune cytopenia is a known and frequent complication of the transplant procedure, particularly in younger patients with non-malignant diseases, such as inherited metabolic disorders. In light of the elevated incidence of autoimmune cytopenias in young patients and patients with Hurler syndrome, we have voluntarily focused enrollment toward pediatric patients older than two years of age diagnosed with leukodystrophies. We plan to continue enrolling patients with leukodystrophies in the study.

Blood Cancers

MGTA-456 has also shown promise in patients with blood cancers, where initial, first-in-human clinical testing of MGTA-456 was performed by Novartis between 2012 and 2016 in adult (n=15) and pediatric (n=3) patients with blood cancers conditioned with a myeloablative protocol, which means near-complete elimination of stem cells in the bone marrow. The study focused on the simultaneous transplant of two cord blood units, or double cord blood transplant, where one unit was expanded using the AHR antagonist to produce MGTA-456 and the other unit was unmanipulated. MGTA-456 successfully engrafted in all 18 patients treated in a median of 14.5 days.

In a subsequent Phase 1/2 study conducted at the University of Minnesota beginning in 2014, nine patients underwent a myeloablative conditioning regimen before receiving MGTA-456 as a single stem cell source, and the other nine patients underwent non-myeloablative conditioning before receiving MGTA-456.

Data from this study were presented at the American Society of Hematology, or ASH, annual meeting in December 2017. MGTA-456 successfully engrafted in all 18 patients, with rapid time to neutrophil engraftment and immune recovery regardless of conditioning intensity. Under the myeloablative protocol, all patients transplanted with MGTA-456 engrafted with a median time to neutrophil recovery of 14 days. None of the patients transplanted with MGTA-456 failed to engraft. Among patients conditioned with the non-myeloablative protocol, all patients treated with MGTA-456 engrafted with a median time to neutrophil recovery of eight days. There were no early

engraftment failures among patients treated with MGTA-456, and no late engraftment failures in any patient treated with MGTA-456, with a patient follow-up of more than two years for all studies. This supports the view that MGTA-456 contains HSCs that retain long-term engraftment. Overall survival at two years for patients treated with MGTA-456 was 67%.

Sickle Cell Disease

We have also explored the possibility of studying MGTA-456 in patients with sickle cell disease. After careful review of comprehensive transplant outcomes data in sickle cell disease from the CIBMTR, we have concluded that the current transplant process will require optimization through targeted conditioning programs before MGTA-456 could deliver maximum clinical benefit to patients with sickle cell disease. We will continue our focus on advancing our potentially transformative conditioning and mobilization programs for patients with sickle cell disease.

Clinical Development Plan

We intend to study MGTA-456 in conditions where we believe it may bring durable and transformative benefit to patients. If the results from the Phase 2 study in IMDs are favorable, we plan to initiate a Phase 3 trial in 2020 in patients with IMDs.

There are approximately 400 patients diagnosed with IMDs each year in the U.S. and Europe only.

We plan to continue to explore MGTA-456 in patients with blood cancers who could benefit from a well-matched stem cell transplant with a high dose of cells. We are in discussions with physician groups and regulatory agencies on the ideal endpoints and comparator treatments to enable clinical trial(s), which could lead to registration. We are adding to the body of data for MGTA-456 in blood cancers through an investigator-initiated Phase 2 study in blood cancers at the University of Minnesota, which was initiated in December 2018. We anticipate that this study will triple the number of patients with blood cancers undergoing myeloablative conditioning treated with MGTA-456 as a stand-alone cell source, build experience with the cryopreserved formulation of MGTA-456 and help inform the design of further Company-sponsored studies in patients with blood cancers.

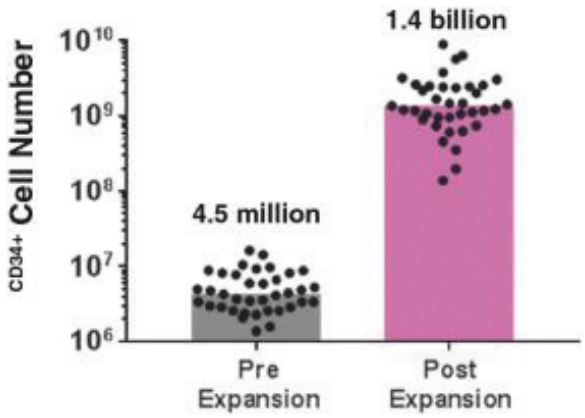
On April 11, 2018, we received orphan drug designation from the FDA for MGTA-456 for the enhancement of cell engraftment in patients receiving stem cell transplant. In addition, we expect to focus on obtaining regenerative medicine advanced therapy, or RMAT, designation, accelerated approval for IMDs and eventually obtain a broad allogeneic cell therapy label.

We obtained the rights to MGTA-456 through an April 2017 license agreement with Novartis granting us the sole worldwide rights to research, develop and commercialize certain AHR antagonist compounds specifically for the expansion of cord blood-derived non-gene-edited/-modified HSCs.

MGTA-456 manufacturing process

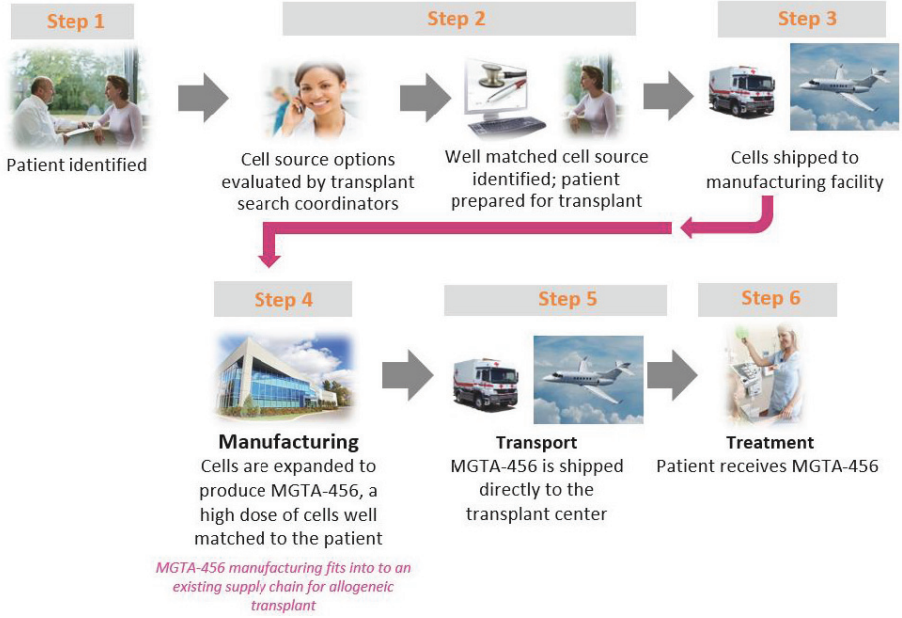
MGTA-456 is manufactured at a GMP-certified contract manufacturing facility, which has produced all batches of MGTA-456 used for clinical trials. The manufacturing process involves selection of CD34+ cells which contain the stem cells, from an umbilical cord blood unit matched to the patient to create a CD34-enriched cell fraction and a CD34-depleted cell fraction. The CD34-depleted fraction is cryopreserved following selection and later thawed and infused at the time of transplant. The CD34-depleted fraction contains immune cells that are important for immune recovery and provide a source of donor T cells that can recognize and kill residual tumor cells in the patient when used in patients with blood cancers. The CD34+ enriched cells are cultured for 15 days with growth factors that promote cell division and the low molecular weight AHR antagonist to produce

MGTA-456. While the cells are being cultured, the patient is conditioned for transplant. The MGTA-456 manufacturing process results in a median increase in the number of CD34+ cells of 327-fold (range: 67-fold to 848-fold) across all studies.



Summary of clinical manufacturing of MGTA-456, CD34+ cell numbers before and after expansion in 36 clinical manufacturing batches

After culture, the cells are washed, characterized, and re-suspended in human serum albumin solution. The cells then undergo quality and release testing. MGTA-456 can then be infused directly into the recipient ("fresh" product) or can be mixed with cryopreservation medium, cryopreserved and infused to the patient after thawing. The ability to cryopreserve MGTA-456 should allow for shipment of the product globally. The manufacturing process takes a total of 15 days from receipt of the cord blood unit to product release. The shelf life of the fresh product is 24 hours after manufacturing. The shelf life of the frozen product is up to 90 days. We are currently working with third-party GMP suppliers for our clinical manufacturing process and with commercial contract manufacturing organizations to develop a process for commercial supply.



Schematic of the MGTA-456 manufacturing process. The cord blood unit is thawed and the $CD34^+$ cells are selected and expanded in the presence of an AHR antagonist. Following expansion, the $CD34^+$ cells are infused followed by the CD34-depleted fraction. The HSCs migrate to the bone marrow and rebuild the blood system.

The manufacturing process for MGTA-456 does not require genomic manipulation. The small molecule AHR antagonist used in the MGTA-456 manufacturing process is manufactured under GMP conditions from readily available starting materials in reliable and reproducible synthetic processes that do not require specialized equipment in the manufacturing process.

Stem cell expansion program: E478

Unmet need

Stem cell transplant with gene-modified HSCs, which is referred to as stem cell gene therapy or genome editing, is a promising treatment approach for a number of inherited diseases but is currently limited by the inability to generate a sufficient dose of gene-modified HSCs to achieve clinically meaningful results.

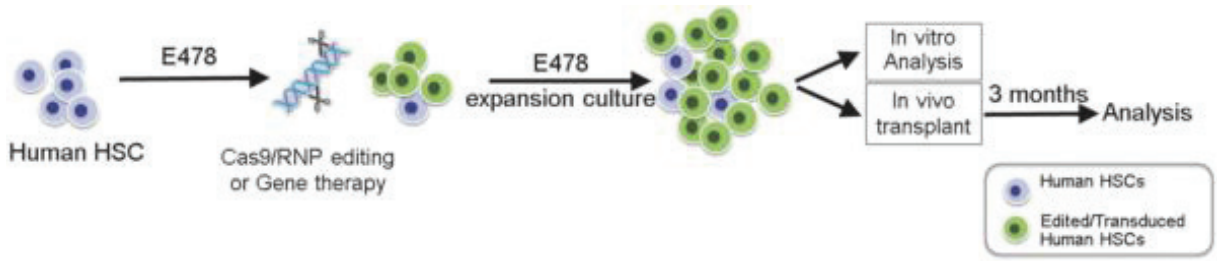
The ability to expand HSCs *ex vivo* has the potential to improve outcomes with gene therapy or genome editing by increasing the dose of genetically modified stem cells. This has been a long-term goal of the field and has the potential to reduce manufacturing costs for these therapies by requiring less viral vector for gene modification of the stem cells. Scaling up vector manufacturing in a cost-effective manner has been a significant challenge for HSC-based gene therapy companies and a significant cost driver. Such cost and capacity issues could limit the commercial viability and widespread deployment of gene therapies. This will only be a bigger challenge as more gene therapies enter the marketplace in the coming years.

Our solution

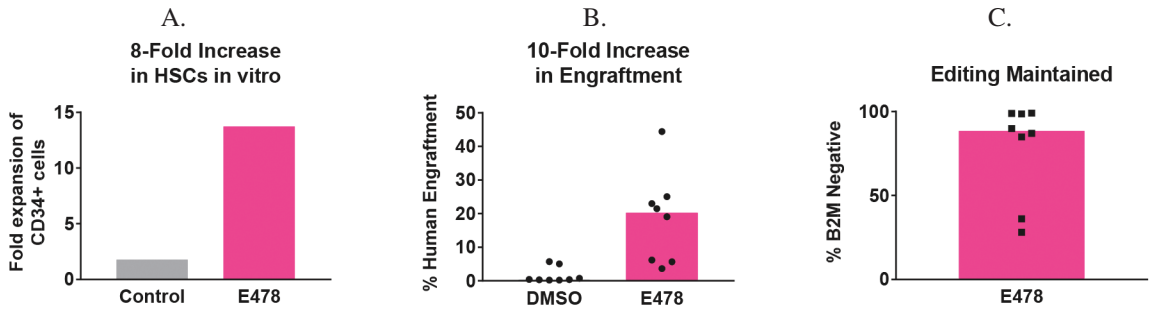
We developed the E478 program in response to an unmet technological need recognized in the field of gene therapy – the challenge of achieving sufficiently high doses of transduced or gene-modified cells. We believe that E478 could represent a key component for unlocking the full value of gene therapy by providing each patient with an optimal dose of gene-modified HSCs for rapid and successful engraftment.

E478 uses the same clinically validated method used to produce MGTA-456, AHR antagonism, to expand HSCs. We are developing E478 specifically to partner with gene therapy and/or genome editing companies to integrate into their manufacturing processes, leading to newly defined cell therapy products.

To demonstrate the utility of E478 for increasing the dose of gene-modified human HSCs that retain engraftment activity, we performed a series of *in vitro* and *in vivo* experiments. We have shown that E478 can generate higher numbers of long-term engrafting cells compared to other expansion technologies. We have presented *in vitro* and *in vivo* data demonstrating successful expansion of gene-modified HSCs from both bone marrow and mobilized blood and *in vivo* engraftment of the expanded cells modified via lentiviral transduction, CRISPR/Cas9 and other gene-modifying approaches.

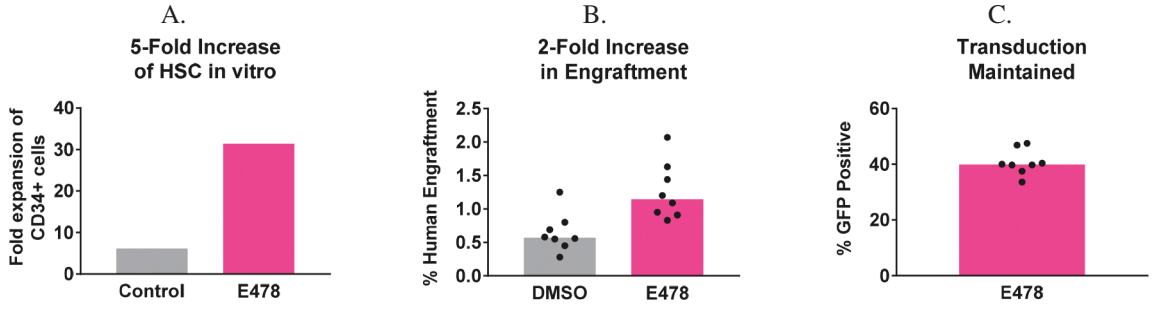


In data we presented at the ASH annual meeting in December 2017, we showed that *in vitro* culture of HSCs with E478 resulted in up to an 8-fold increase in the number of CD34+ cells, a cell population enriched for human HSCs. Transplant of cells expanded with E478 into immune-deficient mice resulted in a 10-fold increase in the number of human cells that engrafted *in vivo* compared to cells cultured in the absence of E478. Importantly, the high levels of editing of the expanded cells obtained during the *in vitro* culture (approximately 80%) were maintained *in vivo*, demonstrating that the expanded and edited HSCs retain engraftment activity.



Expansion and transplantation of edited bone marrow CD34+ cells into NSG mice: Bone marrow-derived CD34+ cells were thawed, edited, and expanded for seven days with (magenta bars) or without (gray bars) E478. **(A)** Fold expansion of CD34+ cells generated over the seven-day culture. **(B)** Human engraftment and **(C)** editing rates in the bone marrow of mice as determined by flow cytometry at 16 weeks post-transplant.

We next performed similar experiments designed to evaluate the ability of E478 to increase HSC numbers for HSC based gene therapy. In these studies, human CD34+ cells isolated from G-CSF mobilized peripheral blood were cultured with cytokines in the presence or absence of E478 and transduced with lentiviral vectors expressing a green fluorescent protein (GFP) to identify cells that were effectively transduced. Inclusion of E478 increased the number of CD34+ cells and improved the level of human cell engraftment while maintaining high levels of transduction.



Expansion and transplantation of transduced peripheral blood derived CD34+ cells into NSG mice: Peripheral-derived CD34+ cells were thawed, transduced, and expanded for seven days with (magenta bars) or without (gray bars) E478. **(A)** Fold expansion of CD34+ cells generated over the seven-day culture. **(B)** Human engraftment and **(C)** editing rates in the peripheral blood of mice as determined by flow cytometry at four weeks post-transplant.

These data demonstrate that E478 can increase the number of gene-modified HSCs that retain the ability to engraft immune deficient mice.

Development plan

We are conducting additional preclinical studies to examine the ability of E478 to expand HSCs transduced with lentiviral vectors.

We are developing E478 specifically to partner with gene therapy and/or genome editing companies to integrate into their manufacturing processes, leading to newly defined cell therapy products.

Post-transplant complications programs

Unmet need

Approximately 40% of all stem cell transplants are allogeneic. GvHD, a reaction that commonly develops after an allogeneic stem cell transplant, occurs when the transplanted cells see the recipient's body as foreign. The grafted cells then attack their new host. It is the result of the donor T cells not matching the recipient's, and this underscores the importance of HLA matching between the donor and the recipient. If they are not well matched, the donor's T cells recognize the recipient's cells as foreign and attack them. Recipients who receive poorly matched stem cells are among those at the highest risk of developing this condition. However, GvHD can occur even with proper HLA matching. Acute GvHD typically occurs within the first 100 days following transplant and can severely damage the skin, liver, and gastrointestinal system. It occurs in approximately 50 to 80% of patients receiving an allogenic stem cell transplant, depending on the specific indication, and accounts for approximately 10% of deaths following an allogeneic transplant. Current treatments for acute GvHD prevention include the prophylactic use of immune suppressive agents that prevent T cell activation, such as cyclosporine or tacrolimus along with mycophenolic acid, first discovered in 1893, which inhibits DNA base synthesis which is required by proliferating T cells or steroids. Despite the use of these powerful immune suppressive agents, most allogeneic transplant patients will experience GvHD. For severe cases, patients are treated with high doses of steroids, immune ablating antibodies or chemotherapy. The use of high-dose non-specific immune suppressive agents for GvHD treatment is correlated with an increased risk of opportunistic and viral infections, poor immune function and is a leading cause of death in allogenic transplant patients.

Our solution

We are developing a unique approach to preventing acute GvHD. Our ADC-based therapeutics are designed to selectively eliminate only the components of the graft that cause acute GvHD, specifically the alloreactive T cells. This ADC therapy is intended to be dosed *in vivo* at the time of transplant and eliminate the activated alloreactive T cells. By specifically targeting the alloreactive T cells that arise shortly after transplant, this therapy should spare the remainder of the immune system to allow immune recovery and protection from opportunistic infections.

Commercialization Plan

We plan to establish sales, marketing, and commercial product distribution capabilities. Ahead of our first commercial launch, we are building upon relationships with transplant centers and thought leaders, furthering our understanding of the influences on the transplant decision-making process, refining our market research into reimbursement and market access, and leveraging our partnership with the National Marrow Donor Program/Be The Match, or NMDP/BTM. Transplants are currently conducted in a small number of specialist sites in the U.S. and Europe. There are approximately 170 transplant centers in the U.S. that are accredited through the NMDP, of which 20% of these transplant centers collectively account for over 50% of transplant volume. In Europe, approximately 205 transplant centers are accredited through the Joint Accreditation Committee ISCT-Europe-EMBT and data suggest that 30% of transplants in Europe between 2012 and 2015 were carried out by 20 centers in France and Spain. All of our product candidates are focused on the transplant physician as the key prescriber and decision maker.

As we advance our development programs in the U.S. and Europe, we will evaluate our sales and marketing resource needs. In advance of approval of our first product in the U.S. and Europe, we plan to build out a dedicated transplant-center-focused sales and marketing organization. We intend to leverage the infrastructure developed for MGTA-456 and MGTA-145 to support commercialization of any additional product candidates in our portfolio for which we gain approval. In addition, we will build upon physicians' familiarity with MGTA-456 and MGTA-145 to accelerate adoption of our other stem cell transplant medicines. As additional product candidates advance through our pipeline, our commercial plans may change. In particular, some of our pipeline assets target autoimmune disease indications, which could potentially require commercial reach that captures referring physicians outside of transplant centers and clinical trial sites.

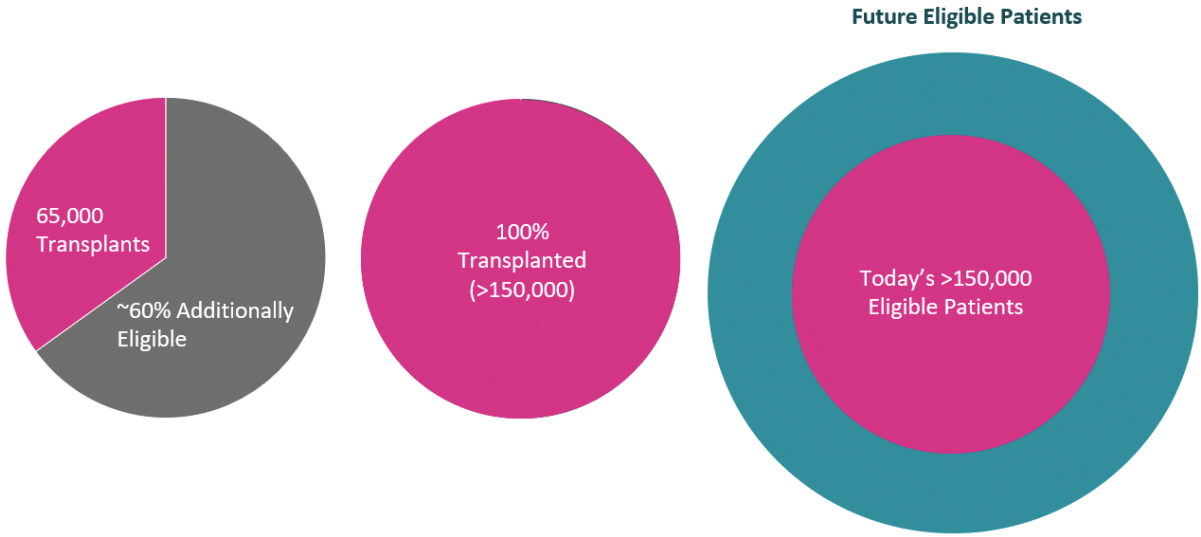
Our commercial strategy may include the use of strategic partners, distributors, a contract sales force or the establishment of our own commercial structure.

Stem cell Transplant Market Opportunity

Despite the risks associated with stem cell transplant, today it is a large market opportunity with approximately 65,000 procedures performed annually. However, this number represents only 40% of the patient population that is eligible for transplant.

At Magenta, we believe our portfolio of product candidates could not only address deficiencies in existing approaches but also extend the curative power of stem cell transplant to more patients. Each of our product candidates contributes uniquely to addressing certain unmet needs in stem cell transplant. By using multiple Magenta products, physicians would be able to tailor the transplant procedure, thereby improving patient outcomes and increasing the potential for every eligible patient to benefit from a stem cell transplant.

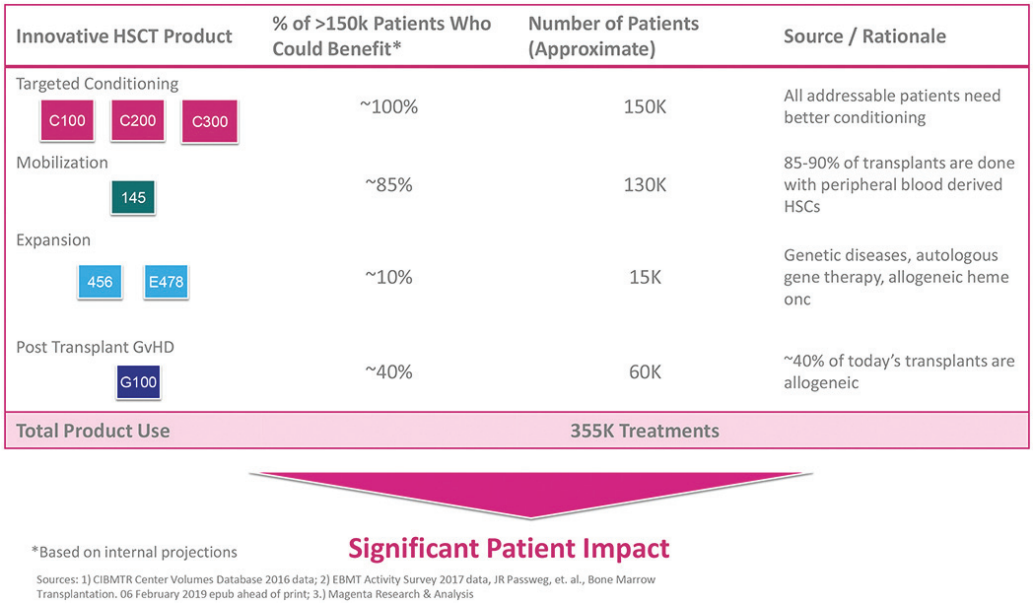
Across diseases where transplant has been shown to result in improved patient outcomes, only a small fraction of eligible patients currently receive a transplant because the risks and challenges outweigh the potential for a cure. Depending on the disease, the barriers for treatment include finding a matched donor, obtaining an adequate cell dose and the morbidity and mortality associated with current conditioning regimens. We believe that by removing the major barriers to transplant with Magenta's programs, we enable transplant for all of the approximately 150,000 eligible patients. Further, by making the procedure less risky and toxic, we believe the eligible patient populations could significantly increase beyond the current numbers. This includes patients with autoimmune diseases, such as multiple sclerosis, where the current risk-benefit of transplant is not considered favorable due to the toxicity of the existing conditioning regimens.



Not To Scale

At Magenta, we are planning to develop our suite of medicines for stem cell transplant across several indications. These include diseases where stem cell transplant is currently a standard of care (e.g., blood cancers such as acute myelogenous leukemia, myelodysplastic syndromes, multiple myeloma, and non-Hodgkin lymphoma), diseases where transplant is performed but limited in use (e.g., hemoglobinopathies such as sickle cell disease and beta-thalassemia), and diseases where the clinical promise for stem cell transplant is emerging (e.g., autoimmune diseases such as systemic sclerosis and multiple sclerosis). The introduction of innovative medicines to the marketplace that address some of the barriers for treatment with transplant would therefore potentially enable each eligible patient across these diseases to receive a transplant, and management's growth rate assumptions, we estimate that the total number of transplants performed in these indications will be approximately 150,000 by 2028 across the U.S. and Europe. Based on this projected growth in transplant volume for these diseases, we further assessed the addressable patient population on a per program basis to estimate the potential number of treatments that could be provided on a per patient basis. This analysis, summarized in the table below, assumes demonstration of clinical outcomes that address barriers to treatment with transplant.

Of the approximately 150,000 estimated stem cell transplant patients in the U.S. and Europe only in approximately 10 years, each will potentially need multiple treatments using products to be developed under our programs:



Autoimmune diseases: emerging data support use of stem cell transplant as a clinically promising one-time therapy, however the high morbidity and mortality associated with current conditioning regimens limit the uptake of transplant as a therapeutic option.

Multiple sclerosis affects approximately 600,000 to 700,000 patients in the U.S. and historically at least 650,000 patients in Europe. Approximately 15,000 new patients in the U.S. and 32,000 new patients in Europe are diagnosed annually. To assess eligibility in this population we focused on the patients with active relapsing-remitting disease and relapsing secondary progressive multiple sclerosis patients who are not adequately treated by current therapies. This population represents approximately 45,000 multiple sclerosis patients who switch therapies each year in the U.S. and we believe that a greater population of patients switch therapies each year in Europe. Many of these patients switch because their current therapy does not adequately control disease activity

such as relapses. Given stem cell transplant's ability to durably eliminate relapses and disease activity in multiple sclerosis patients, we believe a safer transplant procedure would be a viable option for those patients with highly active disease beyond what therapeutics can manage. Currently approximately 1 to 2% of eligible multiple sclerosis patients in the U.S. and Europe receive a stem cell transplant because the risk of the process outweighs the benefits of a potential cure and we believe we can significantly expand this number.

Scleroderma, a chronic connective tissue disease that is characterized by thickening of the skin, is diagnosed in approximately 6,600 patients in the U.S. and 10,900 patients in Europe annually. Approximately 35% (2,300 patients in the U.S., 3,800 patients in Europe) of scleroderma patients suffer from diffuse cutaneous disease and are therefore eligible for stem cell transplant. Currently approximately 1% or less of eligible scleroderma patients in the U.S. and Europe receive a stem cell transplant. However, with the addition of stem cell transplant into the EULAR, treatment guidelines for scleroderma and with the opportunity for a safer transplant procedure, we believe transplant would be a viable option for the severe scleroderma patient population who have no other therapeutic options available.

Our conditioning programs, such as C100 or a combination of C200 and C300, have the potential to provide safer, targeted conditioning for these autoimmune disease patients receiving autologous transplant. We are developing these targeted conditioning approaches to grow the use of transplant in autoimmune disease significantly. In addition, our MGTA-145 product candidate may address the difficult-to-mobilize patients who cannot receive G-CSF because of the risk of triggering autoimmune flares.

Blood Cancers: Stem cell transplant is a standard of care, however a significant number of eligible patients do not receive a transplant because of lack of a matched donor, poor mobilization, and the toxicity of conditioning:

Acute myeloid leukemia, or AML, a cancer arising from myeloid cells, an immature white blood cell found in the bone marrow, is diagnosed in approximately 20,000 patients in the U.S. and 27,000 patients in Europe annually. Approximately 60% (12,500 patients in the U.S., 16,400 patients in Europe) of newly diagnosed patients have a complete response to induction chemotherapy and become eligible for transplant for a more durable remission. However, the current challenges of stem cell transplant and patient co-morbidities limit transplant to approximately 30 to 40% of those patients who are otherwise eligible.

Myelodysplastic syndrome, or MDS, occurs when the blood-forming cells in the bone marrow become abnormal, leading to low numbers of one or more types of blood cells. It is diagnosed in approximately 21,000 patients in the U.S. and 13,300 patients in Europe annually. Approximately 30% (6,700 patients in the U.S., 4,600 patients in Europe) of newly diagnosed patients have high risk disease and are eligible for stem cell transplant. However, toxicities of stem cell transplant and patient co-morbidities limit transplant to approximately 16 to 40% of those patients who are otherwise eligible.

Non-Hodgkin lymphoma, or NHL, a cancer arising from lymphocytes, a type of white blood cell, is diagnosed in approximately 72,000 patients in the U.S. and 95,000 patients in Europe annually. Non-Hodgkin lymphoma, the most common blood cancer, can be treated by autologous stem cell transplant. The largest Non-Hodgkin lymphoma subtype is Diffuse Large B-Cell Lymphoma, or DLBCL, comprising approximately 33% of Non-Hodgkin lymphoma. First-line chemotherapy and targeted therapies like rituximab are effective for about two-thirds of DLBCL patients. In the remaining one-third of patients with relapsed/refractory disease, the National Comprehensive Cancer Network recommends autologous stem cell transplant as one therapeutic option. Therefore, approximately 6,500 newly diagnosed patients in the U.S. and 8,500 patients in Europe are eligible for stem cell transplant. Currently, approximately 60 to 80% of those eligible Non-Hodgkin lymphoma patients in the U.S. and Europe receive a stem cell transplant.

Multiple myeloma, or MM, a cancer arising from plasma cells, is diagnosed in approximately 30,000 patients in the U.S. and 40,000 patients in Europe annually. Multiple myeloma represents the second most common blood cancer treated by autologous stem cell transplant. Approximately 50% (15,000 patients in the U.S., 20,000 patients in Europe) of newly diagnosed patients are eligible for stem cell transplant. The National Comprehensive Cancer Network recommends stem cell transplant for patients after primary therapeutic treatment, including novel orals, injectables, and chemotherapy. This recommendation is founded on evidence that autologous stem cell transplant consistently achieves higher response rates and survival rates than primary treatment alone. However, toxicities of stem cell transplant and patient co-morbidities limit transplant to approximately 55 to 60% of those patients who are otherwise eligible.

Our conditioning programs, such as C100 and C200, have the potential to provide safer, targeted conditioning for these blood cancer patients who are too frail to tolerate the current conditioning regimens. Our MGTA-145 product candidate has the potential to address the difficult-to-mobilize patients who are suffering from blood cancers as well so that patients can access a robust single-day mobilization regimen. For allogeneic transplant recipients, MGTA-456 offers access to a well-matched cell therapy, with a high stem cell dose and the higher likelihood of durable clinical outcomes.

Hemoglobinopathies: Stem cell transplant is clinically accepted but still limited in use due to challenges identifying well-matched donors and conditioning-associated morbidity and mortality. Gene therapy is a promising treatment option but is limited by the same conditioning challenges as well as the ability to obtain a sufficient dose of gene-modified cells.

Sickle cell disease, or SCD, is diagnosed in approximately 1,700 patients in the U.S. and 720 patients in Europe annually. Approximately 25% (400 patients in the U.S., 180 patients in Europe) of patients with sickle cell disease have severe disease and are therefore eligible for stem cell transplant. Currently, only approximately 36 to 58% of eligible patients with sickle cell disease in the U.S. and Europe receive a stem cell transplant because it is particularly difficult to find a matched donor and the risk of the procedure often outweighs the benefits of a potential cure.

Beta-thalassemia is diagnosed in approximately 500 patients in the U.S. and 1,500 patients in Europe annually. Approximately 60% (340 patients in the U.S., 1,020 patients in Europe) of patients with beta-thalassemia patients have severe disease, or beta-thalassemia major, and are therefore eligible for stem cell transplant. Currently, only approximately 27% of eligible beta-thalassemia patients in the U.S. and Europe receive a stem cell transplant because they are unable to find a matched donor and/or the risk of the process outweighs the benefits of a potential cure.

Our conditioning programs, such as C200, have the potential to provide safer, targeted conditioning for these hemoglobinopathy patients receiving autologous gene therapy or an allogeneic transplant. Our MGTA-145 product candidate has the potential to address patients with sickle cell disease who cannot receive G-CSF because of the risk of triggering sickle cell crises. E478 uses the same clinically-validated mechanism as MGTA-456 to generate higher doses of gene-modified stem cells.

Our portfolio of products is designed to address several unmet needs in transplant, providing the potential opportunity for our products to be used in combination to create disease-tailored solutions for patients. Based on the projected growth in transplant volume of approximately 150,000 transplants for the U.S. and Europe above, and management assumptions, we estimate that, by 2028 the approximately 150,000 stem cell transplant patients could be treated by around 355,000 aggregate treatments per year. This estimate includes approximately 150,000 treatments under our conditioning programs, 130,000 treatments under our mobilization program, 15,000 treatments under our expansion programs, and 60,000 treatments under our post-transplant complications program, each on an annual basis. Therefore, in 2028 an estimated 1.8 products could be used on a per patient basis annually.

Manufacturing

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently depend on third-party contract manufacturing organizations, or CMOs, for all of our requirements of raw materials, drug substance and drug product for our preclinical research and our ongoing clinical trial of MGTA-456. We have not entered into long-term agreements with our current CMOs. We intend to continue to rely on CMOs for later-stage development and commercialization of MGTA-456, as well as the development and commercialization of any other product candidates that we may identify. Although we rely on CMOs, we have personnel and consultants with extensive manufacturing experience to oversee the relationships with our contract manufacturers.

We believe the synthesis of the AHR antagonist that we use to manufacture MGTA-456 is reliable and reproducible from readily available starting materials, and the synthetic routes are amenable to large-scale production and do not require unusual equipment or handling in the manufacturing process. We acquired data from Novartis related to the chemical synthesis of the AHR antagonist, which has been manufactured by Novartis to satisfy our immediate and near term clinical and preclinical needs. We also acquired data from Novartis related to manufacturing of MGTA-456, which is currently manufactured by a single CMO to satisfy our immediate and near term clinical and pre-commercial needs. Drug product formulation optimization work for MGTA-456 is also in progress with this CMO. We have begun to engage with a second drug product manufacturer for our commercial supply needs for MGTA-456.

Our manufacturing strategy enables us to more efficiently direct financial resources to the research, development, and commercialization of product candidates rather than diverting resources to internally develop manufacturing facilities. As our product candidates advance through development and commercialization, we expect to enter into longer-term commercial supply agreements with key suppliers and manufacturers to fulfill and secure the ongoing and planned preclinical, clinical, and, if our product candidates are approved for marketing, commercial supply needs for ourselves and our collaborators.

Manufacturing of any product candidate is subject to extensive regulations that impose various procedural and documentation requirements, which govern recordkeeping, manufacturing processes and controls, personnel, quality control and quality assurance, among others. We expect that all of our CMOs will manufacture MGTA-456 under current Good Manufacturing Practice, or cGMP, conditions. cGMP is a regulatory standard for the production of pharmaceuticals to be used in humans.

Competition

The biotechnology industry is extremely competitive in the race to develop new products and treatment modalities. While we believe we have significant competitive advantages with our industry-leading expertise in transplant medicine, preclinical and clinical development expertise, our comprehensive approach to patient care and intellectual property position, we may face competition for our development programs from companies focused on traditional therapeutic modalities, such as small molecules and antibodies, as well as companies developing next-generation cell therapies. Competition is likely to come from multiple sources, including larger pharmaceutical companies, biotechnology companies and academia. For any products that we may ultimately commercialize, not only will we compete with any existing therapies and those therapies currently in development, we will have to compete with new therapies that may become available in the future. We believe we are the only company that is committed to addressing all of the major limitations and challenges of stem cell transplant to revolutionize an entire field of medicine. We are building a comprehensive portfolio of novel therapeutic development programs to address all major unmet medical needs inherent to the existing stem cell transplant process, which distinguishes us from our competition.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and product marketing than

we currently do. 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. 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.

Stem cell transplant is used to treat a number of diseases and indications. We are aware of a number of companies that are developing therapeutics, including biologics, small molecules and CAR T therapies that are directed to the treatment of autoimmunity, blood cancers, and genetic diseases that overlap with certain current stem cell transplant indications. The following competitive overview is focused on companies that are developing technologies to improve the distinct steps of stem cell transplant.

Competitors in our stem cell expansion programs include the following:

- Gamida Cell Ltd., which is developing a UCB-derived cell product that uses a small molecule to inhibit differentiation and enhance functionality of *ex vivo*-expanded HSCs;
- Nohla Therapeutics, Inc., which is developing a method of *ex vivo* expansion of cord blood stem/progenitor cells resulting in "off-the-shelf" universal donor cellular therapies;
- ExCellThera Inc., which is focused on *ex vivo* expansion of stem cells using a pyrimidoindole-derivative small molecule;
- Angiocrine Bioscience, Inc., which is expanding cord blood and gene-modified HSCs using an endothelial cell feeder layer; and
- Intellia Therapeutics, Inc., which has exclusively licensed from Novartis the AHR antagonist that we use to manufacture MGTA-456 for expansion of gene-modified HSCs only.

Competitors in our conditioning programs include the following:

- Actinium Pharmaceuticals, Inc., which is developing an antibody to CD45 that is linked to radioisotope iodine-131;
- Stanford University, which is developing an antibody to CD117 that is not conjugated to any toxin;
- Forty Seven, Inc., which is developing an antibody to CD117 that is not conjugated to any toxin and is used in combination with an antibody to CD47; and
- Molecular Templates Inc., which is developing an antibody to CD45 that is conjugated to engineered Shiga-toxin.

Competitors in our post-transplant complications programs (acute GvHD) include the following:

- Bellicum Pharmaceuticals, Inc., which is developing a combination of genetically modified T cells and activator agent rimiducid;
- Kiadis Pharma NV, which is developing a single dose donor lymphocyte infusion with functional, mature immune cells from a partially matched family member; and
- Abbvie Inc., which has a Bruton's tyrosine kinase (BTK) inhibitor that is approved for use in chronic GvHD.

Competitors in our mobilization programs include the following:

- BioLineRx Ltd., which is developing BL-8040, a peptide that functions as a high-affinity antagonist for CXCR4.

Licenses and Collaborations

Alliance with Novartis

In April 2017, we entered into a license agreement with Novartis International Pharmaceutical Ltd., or Novartis, pursuant to which Novartis granted us an exclusive, worldwide, sublicensable license to research, develop and commercialize certain licensed products that contain Novartis compounds for the expansion of cord blood derived non-Gene-Edited/-Modified HSCs. We refer to this agreement as the Novartis Agreement. The license granted to us under the Novartis Agreement is subject to certain rights retained by Novartis for internal research purposes and certain third parties for research and educational purposes. Certain of the rights licensed to us under the Novartis Agreement are also subject to any retained rights of the U.S. government in the licensed patents. The Novartis Agreement led to the establishment of MGTA-456 as one of our programs. Under the terms of the Novartis Agreement, we are responsible for all research, development, regulatory and commercialization activities related to licensed products. We are required to use commercially reasonable efforts to develop and commercialize licensed products in the U.S., United Kingdom, France, Germany, Spain, Italy and Japan.

Pursuant to the Novartis Agreement, we issued to Novartis 2,500,000 shares of Series A preferred stock and 643,550 shares of Series B preferred stock. We will be required to make milestone payments to Novartis upon dosing the first patient in BLA-enabling clinical trials and upon regulatory approvals of licensed products across ultra-orphan, hemoglobinopathies and other indications. For ultra-orphan indications, we may be required to pay development and regulatory milestones of up to $13.0 million for each of the first two indications, and up to $5.0 million for a third indication. For hemoglobinopathies, we may be required to pay development and regulatory milestones of up to $13.0 million per indication, for the first two indications. For all other indications that are not an ultra-orphan or hemoglobinopathy, we may be required to pay development and regulatory milestones of up to $75.0 million for the first indication and up to $45.0 million for the second indication. Across all licensed products, we may be required to pay Novartis up to $125.0 million in sales milestones, based on the first achievement of certain aggregate worldwide annual net sales thresholds. We are also required to pay tiered royalties to Novartis on worldwide net sales of licensed products by us, ranging from low single-digit percentages to up to 20% for higher net sales, which royalties are potentially subject to reduction and offset during the royalty term. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the later of (i) expiration of the last valid claim of a licensed patent right that covers the manufacture, use or sale of such licensed product, or the licensed compound used in the manufacture of such licensed product, in such country and (ii) ten years following the first commercial sale of such licensed product in such country.

Novartis controls the filing, prosecution, maintenance and enforcement of the licensed patent rights at its expense. We have the right to assume these responsibilities should Novartis not wish to pursue them. We own all rights in any intellectual property related to the licensed compound or licensed products that we solely develop under the Novartis Agreement. The Novartis Agreement does not otherwise allocate ownership of improvements developed thereunder; however, Novartis grants us a non-exclusive, royalty-free license to practice any improvements that Novartis owns under the Novartis Agreement in connection with non-gene-edited/-modified HSCs, and we grant a non-exclusive, royalty-free license to Novartis to practice any improvements that we own under the agreement outside of such field.

The Novartis Agreement will continue until the last-to-expire royalty term for a licensed product unless terminated earlier by either party. Each party may terminate the Novartis Agreement due to the other party's insolvency or uncured breach of a material obligation. We have the right to terminate the Novartis Agreement in its entirety or on a product-by-product or country-by-country basis for convenience upon 90 days' prior written notice to Novartis. Upon termination of the Novartis Agreement by us for convenience or by Novartis for cause, the license granted to us by Novartis will terminate and we will grant a worldwide, perpetual, non-exclusive license to Novartis to develop and commercialize the licensed products under any intellectual property that we (i) control and used in the development, manufacture or commercialization of licensed products or (ii) developed under the agreement to develop and commercialize the licensed products.

Collaboration with Be The Match BioTherapies

In November 2017, we formalized a collaboration agreement with Be The Match BioTherapies, or BTMB, which we refer to as the BTMB Agreement, to advance our relationship developed under a Memorandum of Understanding executed in April 2017. Pursuant to the BTMB Agreement, BTMB grants us priority access to subject matter experts at the National Marrow Donor Program/Be The Match, or NMDP/BTM, the CIBMTR, which is an affiliation between the Medical College of Wisconsin and NMDP/BTM, and BTMB for consultation on our clinical development and commercialization needs. The parties may enter into additional riders to the BTMB Agreement from time to time to expand the services that BTMB provides thereunder.

We believe that, through this priority access, the partnership enables us to establish relationships across transplant centers and with transplant physicians, giving us access to clinical strategy and development operational support, and potentially supporting our eventual commercialization efforts across several programs.

Under the BMTB Agreement, we will make quarterly payments to BTMB of $25,000. The term of the BTMB Agreement will continue until December 31, 2020 unless terminated earlier by either party. Either party may terminate the BTMB Agreement in whole or in part (i) for convenience upon 60 days' notice, (ii) for the other party's uncured material breach upon ten days' notice or (iii) upon the other party's insolvency.

*Center for International Blood & Marrow Transplant Research

Harvard University License Agreement

In November 2016, we entered into a license agreement with Harvard University, or Harvard, pursuant to which Harvard granted us the worldwide exclusive, subject to Harvard's retained right solely for research and educational purposes, sublicensable, right, to research, develop and commercialize licensed products under certain conditioning-related and mobilization-related patents. The license granted to us under the Harvard Agreement is also subject to any retained rights of the U.S. government in the licensed patents. Under the terms of the agreement, which we refer to as the Harvard Agreement, we will be responsible for all research, development, regulatory and commercialization activities related to licensed products. We are obligated to use commercially reasonable efforts to commercialize at least two licensed products under the Harvard Agreement, including one for conditioning and one for mobilization. The license from the Harvard Agreement relates to our C100, C200 and mobilization programs.

Pursuant to the Harvard Agreement, we made an upfront payment to Harvard of $85,000 and issued to Harvard and the other co-owners of the licensed patent rights (The General Hospital Corporation d/b/a Massachusetts General Hospital, and Children's Medical Center Corporation) 385,063 shares of our common stock. In addition, we reimbursed Harvard for approximately $300,000 in expenses incurred by Harvard in connection with the licensed patent rights. Harvard is also entitled to receive an annual license maintenance fee of $25,000 for each calendar year through 2019 and $50,000 for each calendar year thereafter until expiration or termination of the Harvard Agreement.

Harvard is entitled to payments upon certain development and regulatory milestones for the first two licensed products of up to $7.4 million per licensed product. In addition, we must pay Harvard low-single digit royalties on net sales of licensed products. If we or our affiliates or sublicensees under the Harvard Agreement commence a legal action to challenge the validity, enforceability or scope of any licensed patents, the royalty rate payable to Harvard will double during the pendency of such proceeding and will remain double thereafter if such action is determined in Harvard's favor. Depending on the type of licensed product, royalties are payable on a product-by-product and country-by-country basis until the later of (i) the last to expire valid claim in the applicable country covering or claiming the composition, manufacture, sale or use of such licensed product and (ii) 12 years from the date of the first commercial sale of such licensed product in such country.

Harvard controls the filing, prosecution and maintenance of the licensed patent rights at our expense. We have the first right, but not the obligation, to enforce licensed patent rights against third-party infringement.

The term of the Harvard Agreement will continue until the later of (i) the expiration of the last to expire valid claim under a licensed patent, and (ii) the expiration of the last royalty period Each party has the right to terminate the Harvard Agreement due to the other party's uncured material breach or insolvency. In particular, Harvard may terminate the Harvard Agreement upon our uncured failure to meet certain development and regulatory milestone deadlines set forth therein. We have the right to terminate the Harvard Agreement for convenience upon 60 days' prior written notice to Harvard. Upon termination of the Harvard Agreement for any reason, the license granted to us by Harvard will terminate.

Research, Development Option and License Agreement with Heidelberg Pharma Research GmbH

In March 2018, we entered into an exclusive research, development option and license agreement with Heidelberg Pharma Research GmbH, or Heidelberg Pharma. We refer to this agreement as the Heidelberg Agreement. Heidelberg Pharma has developed a proprietary antibody targeted amanitin conjugates platform. This collaboration enables our research and development efforts across several targeted conditioning programs through the combination of our proprietary antibodies and Heidelberg Pharma's antibody targeted amanitin conjugates platform.

Under the terms of the Heidelberg Agreement, Heidelberg Pharma has granted to us a worldwide, non-exclusive research license for a one-year period with respect to certain targets set forth in an agreed-to research plan. We have the option to extend such license for up to an additional three years. In December 2018, we exercised this option to extend such license for one year. We also have an option to obtain an exclusive target-specific research license, which would expire two years after the exercise of such option. We have exercised this option for two initial targets with signature of the Heidelberg Agreement. In addition, we will have an option to obtain a target-specific exclusive license for global development and commercialization rights to each of the product candidates resulting from the research collaboration. We may obtain such exclusive target-specific rights to up to four targets. In October 2018, we exercised one of the target-specific development options. We are required to use commercially reasonable efforts to perform our research activities under the Heidelberg Agreement and, if we exercise our right to obtain a development and commercialization license, we are required to use commercially reasonable efforts to pursue development and commercialization of a product directed to the applicable target.

Under the terms of the Heidelberg Agreement, we granted Heidelberg Pharma a worldwide, non-exclusive license under all of our patents and know-how, and any improvements of the foregoing developed under the Heidelberg Agreement, that are reasonably necessary or useful for Heidelberg Pharma to perform its research activities under the Heidelberg Agreement. In addition, we grant Heidelberg Pharma a worldwide, royalty-free, non-exclusive license under all joint improvements developed under the Heidelberg Agreement for non-clinical research purposes only.

Payment terms to Heidelberg Pharma include an upfront technology access fee, research exclusivity fees with respect to the two initial targets, and payments for research support. Heidelberg Pharma is entitled to additional fees of between $50,000 and $1.1 million in the aggregate if we extend the initial research license or if we exercise our research exclusivity options with respect to additional targets. Upon our exercise of an option for an exclusive development and commercialization license, with respect to a target, we are required to make a low single digit million-dollar payment to Heidelberg Pharma for each exercised option. In addition, on a per target basis, we may be required to pay development, regulatory and commercial milestones totaling up to approximately $83.5 million per target. We will pay Heidelberg Pharma mid-single digit royalties on a country-by-country and product-by-product basis, on worldwide net product sales of licensed products. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the later of (i) expiration of the last valid claim of a licensed patent right that covers the use, import, offering for sale, or sale of such licensed product in such country, and (ii) ten years following the first commercial sale of such licensed product in such country. We have the option to buy-down royalties at certain points during the development path of each product.

Heidelberg Pharma will own all improvements solely related to the intellectual property rights Heidelberg Pharma licensed to us under the Heidelberg Agreement. We will own all improvements solely related to the intellectual property rights that we licensed to Heidelberg Pharma and all other intellectual property rights developed under the Heidelberg Agreement for which ownership is not otherwise allocated.

Heidelberg Pharma controls the filing, prosecution, maintenance and enforcement of the intellectual property that it licenses to us under the Heidelberg Agreement. We have the right to enforce such licensed intellectual property against infringement if the infringement is competitive with our licensed products and Heidelberg Pharma does not pursue enforcement. We control the filing, prosecution, maintenance and enforcement of the intellectual property we license to Heidelberg Pharma under the Heidelberg Agreement.

The term of the Heidelberg Agreement will continue until the last to expire royalty term unless terminated earlier by either party. Each party has the right to terminate the Heidelberg Pharma Agreement due to the other party's uncured material breach or insolvency on a product-by-product or target-by-target basis. We have the right to terminate the Heidelberg Agreement for convenience in its entirety or on a product-by-product, target-by-target or country-by-country basis upon 60 days' prior written notice to Heidelberg Pharma if terminating before the first commercial sale of a product in a country or upon six months' prior written notice to Heidelberg Pharma if terminating after the first commercial sale of any product directed to such target in such country.

Upon termination of the Heidelberg Agreement in its entirety or with respect to a product or target, all applicable licenses granted to us will terminate immediately.

Bachem Master Development and Manufacturing Agreement

In February 2018, we entered into a Master Development and Manufacturing Agreement with Bachem Americas, Inc., or Bachem. This agreement, which we refer to as the Bachem Agreement, governs several projects related to the development and manufacture of CXCR2 agonists, including MGTA-145, each pursuant to a separate project plan. The active pharmaceutical ingredient of MGTA-145 is a 69 amino acid protein. We selected Bachem as our contract manufacturer for this program based on their deep expertise in the synthesis and

production of proteins. Financial terms related to this agreement will be determined on a project-by-project basis. In April 2018, we entered into an initial one-year project plan pursuant to which Bachem will be responsible for producing batches of MGTA-145 for GLP toxicology studies completed in 2018, as well as GMP material for the Phase 1 study of MGTA-145, which is expected to commence in the first half of 2019.

The term of the Bachem Agreement is initially five years and will be automatically renewed for one-year periods unless either party provides the other with written notice of nonrenewal at least three months prior to expiry. Each party may terminate the agreement upon a material uncured breach of the other party. During the term, Bachem will be restricted from producing a pre-defined set of agonists, including MGTA-145, for clinical or commercial use by any third party without our prior written consent, as long as Bachem remains our primary supplier of CXCR2 agonists. Each project plan may be terminated independently of the agreement as a whole.

Clinical Trial Agreement with University of Minnesota

In January 2018, we entered into a Clinical Trial Agreement with the Regents of the University of Minnesota, or UMinn, pursuant to which UMinn will undertake a Phase 2 clinical trial with MGTA-456 for the treatment of inherited metabolic diseases. Under this agreement, UMinn is also responsible for the manufacture and supply of the required quantities of MGTA-456 for the trial, subject to specified quality assurance provisions.

The term of the agreement will run through the course of the trial, unless earlier terminated. Each party may terminate the agreement immediately upon the other party's material failure to follow the specified protocol for the trial or upon a material uncured breach by the other party.

Intellectual Property

Overview

We strive to protect the proprietary product candidates and technologies that we believe are important to our business, including seeking and maintaining patent protection intended to cover the composition of matter of our product candidates, their methods of use, related technologies, diagnostics and other inventions. As of February 28, 2019, our owned patent portfolio is composed of two U.S. patents, two Australian patents, approximately 60 pending U.S. and foreign patent applications, and more than 45 pending U.S. provisional patent applications. In addition, we have licensed more than 160 patents and pending patent applications in the U.S. and foreign jurisdictions.

Patent Rights Relating to Our Targeted Conditioning Program

Our C200 patent portfolio includes three patent families that we own. As of February 28, 2019, the first family, directed to compositions and methods for the depletion of CD117+ cells, includes one issued U.S. patent, one pending U.S. patent application, one granted Australian patent, and 13 pending patent applications in foreign jurisdictions. The issued U.S. patent, the granted Australian patent, and any other patents that grant in this first family would be expected to expire in 2037, absent any applicable patent term extensions. As of February 28, 2019, the second family, directed to compositions and methods for the depletion of CD117+ cells, includes one pending U.S. patent application and two pending Patent Cooperation Treaty, or PCT, patent applications. As of February 28, 2019, the third family, directed to compositions and methods for the depletion of CD117+ cells, includes two pending PCT patent applications and one pending patent application in each of the U.S., Argentina, and Taiwan. Any patents that grant from these two families would be expected to expire in 2038, absent any applicable patent term extensions.

Our C100 patent portfolio includes two patent families that we own. As of February 28, 2019, the first family, directed to compositions and methods for the depletion of cells, includes two pending U.S. patent

applications, one granted Australian patent, and 13 pending patent applications in foreign jurisdictions. The Australian patent and any other patents that grant from this family would be expected to expire in 2037, absent any applicable patent term extensions. As of February 28, 2019, the second family, directed to anti-CD45 antibodies and conjugates thereof, includes approximately 30 pending U.S. provisional patent applications. Any patents that grant from applications claiming priority to these provisional applications would be expected to expire in 2039, absent any applicable patent term extensions.

Our C300 patent portfolio includes two patent families that we own. As of February 28, 2019, the first family, directed to compositions and methods for the depletion of cells, includes one pending PCT application. Any patents that grant from this family would be expected to expire in 2038, absent any applicable patent term extensions. As of February 28, 2019, the second family, directed to methods of conditioning patients undergoing CAR immunotherapy, includes two pending U.S. provisional patent applications. Any patents that grant from applications claiming priority to these provisional applications would be expected to expire in 2039, absent any applicable patent term extensions.

In addition, we have licensed two patent families from Harvard directed to compositions and methods for non-myeloablative conditioning. As of February 28, 2019, these families include one allowed U.S. patent application, one pending U.S. patent application, one pending PCT patent application, and more than ten pending patent applications in foreign jurisdictions. Any patents that grant from these families would be expected to expire between 2036 and 2037, absent any applicable patent term extensions.

We have also licensed patent rights from Heidelberg Pharma that include four patent families directed to amatoxin conjugates, methods of treatment, and methods of synthesizing amatoxins. As of February 28, 2019, these families include more than 85 granted patents and pending patent applications in jurisdictions worldwide. The granted patents and any other patents that grant from these families would be expected to expire between 2030 and 2038, absent any applicable patent term extensions.

Patent Rights Relating to Our Stem Cell Mobilization Program

We own two patent families directed to methods of mobilizing HSCs. As of February 28, 2019, the first family includes one issued U.S. patent, one pending U.S. patent application, and one pending PCT patent application. The issued U.S. patent would be expected to expire in 2037, absent any applicable patent term extensions, and any other patents that grant in this first family would be expected to expire between 2037 and 2038, absent any applicable patent term extensions. As of February 28, 2019, the second family includes one pending U.S. provisional patent application, and any patents that grant from applications claiming priority to this provisional application would be expected to expire in 2039, absent any applicable patent term extensions.

In addition, we have licensed two patent families from Harvard. As of February 28, 2019, the first family, directed to methods and compositions for mobilizing HSCs, includes one pending U.S. patent application and eight pending patent applications in foreign jurisdictions. Any patents that grant from this family would be expected to expire in 2034, absent any applicable patent term extensions. As of February 28, 2019, the second family, directed to highly engraftable hematopoietic stem cells, includes one pending U.S. patent application and pending patent applications in Europe and Japan. Any patents that grant from this family would be expected to expire in 2037, absent any applicable patent term extensions.

Patent Rights Relating to Our Stem Cell Expansion Program

With regard to our clinical candidate, MGTA-456, we have licensed one patent family from Novartis directed to AHR antagonists and their use in the expansion of HSCs. As of February 28, 2019, this family includes two issued U.S. patents, one with method claims covering the use of AHR antagonists in the expansion of HSCs and one with composition of matter claims covering AHR antagonists, one pending U.S. patent application, and more than 50 granted patents and pending patent applications in jurisdictions worldwide. The

issued U.S. patents are expected to expire in 2032 and 2031, respectively, absent any applicable patent term extensions. The granted foreign patents and any patents that may grant from U.S. and foreign pending patent applications in this family would be expected to expire in 2029, absent any applicable patent term extensions.

Our E478 patent portfolio includes three patent families that we own. As of February 28, 2019, the first family includes one pending PCT patent application, two pending U.S. patent applications, and pending patent applications in Argentina and Taiwan with composition of matter claims covering E478. Any patents that grant from this family would be expected to expire in 2038, absent any applicable patent term extensions. As of February 28, 2019, the second family includes one pending U.S. patent application and one pending PCT patent application with method claims covering expansion of gene-edited HSCs. Any patents that grant from this family would be expected to expire in 2038, absent any applicable patent term extensions. As of February 28, 2019, the third family includes one pending U.S. provisional patent application relating to our E478 program. Any patents that grant from applications claiming priority to this provisional application would be expected to expire in 2039, absent any applicable patent term extensions.

We also own three patent families directed to methods of treatment that are applicable to both our MGTA-456 and E478 programs. As of February 28, 2019, the first family includes one pending U.S. patent application and one pending PCT patent application, the second family includes one pending PCT patent application, and the third family includes one pending U.S. provisional patent application. Any patents that grant from these families would be expected to expire between 2038 and 2039, absent any applicable patent term extensions.

Patent Rights Relating to Our GvHD Program

We own three patent families directed to compositions and methods for treating GvHD. As of February 28, 2019, the first family includes one pending PCT patent application, two pending U.S. patent applications, and pending patent applications in Argentina and Taiwan. Any patents that grant in this first family would be expected to expire in 2038, absent any applicable patent term extensions. The second family includes one pending PCT patent application, and any patents that grant from this patent application would be expected to expire in 2039, absent any applicable patent term extensions. The third family includes a pending U.S. provisional patent application, and any patents that grant from applications claiming priority to this U.S. provisional patent application would be expected to expire in 2039, absent any applicable patent term extensions.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries in which we have filed, including the U.S., the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the U.S., a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a patent that covers a drug or biological product may also be eligible for patent term extension when FDA approval is granted for a portion of the term effectively lost as a result of the FDA regulatory review period, subject to certain limitations and provided statutory and regulatory requirements are met. Any such patent term extension can be for no more than five years, only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. We may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. In the future, if and when our product candidates receive approval from the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents we may obtain in the future covering those products, depending upon the length of the clinical trials for each product and other factors. There can be no assurance that any of our pending patent applications will issue or that we will benefit from any patent term extension or favorable adjustment to the term of any of our patents.

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify our proprietary and intellectual property position for our product candidates will depend on our success in obtaining effective patent claims and enforcing those claims if granted. However, our owned and licensed pending patent applications, and any patent applications that we may in the future file or license from third parties may not result in the issuance of patents. We also cannot predict the breadth of claims that may be allowed or enforced in our patents. Any issued patents that we may receive in the future may be challenged, invalidated, infringed or circumvented. In addition, because of the extensive time required for clinical development and regulatory review of a product candidate we may develop, it is possible that, before any of our product candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby limiting the protection such patent would afford the respective product and any competitive advantage such patent may provide. For more information, see the section entitled "Risk Factors—Risks Related to Intellectual Property".

Other IP Rights

In addition to patents, we rely upon unpatented trade secrets and know-how and continuing technological innovation to develop and maintain our competitive position. However, trade secrets and know-how can be difficult to protect. We seek to protect our proprietary information, in part, by executing confidentiality agreements with our collaborators and scientific advisors, and non-competition, non-solicitation, confidentiality, and invention assignment agreements with our employees and consultants. We have also executed agreements requiring assignment of inventions with selected scientific advisors and collaborators. The confidentiality agreements we enter into are designed to protect our proprietary information and the agreements or clauses requiring assignment of inventions to us are designed to grant us ownership of technologies that are developed through our relationship with the respective counterparty. We cannot guarantee, however, that we have executed such agreements with all applicable counterparties, such agreements will not be breached, or that these agreements will afford us adequate protection of our intellectual property and proprietary rights. For more information, see the section entitled "Risk Factors—Risks Related to Intellectual Property".

Trademarks

We have filed and obtained trademark protection for the MAGENTA THERAPEUTICS character mark and service mark logo for pharmaceutical research and development services and biochemical research and development services. We have also filed for trademark protection for the #THECOLOROFCURE character mark for promoting public awareness of medical disorders and their treatment, promoting public awareness of bone marrow diseases, cancer, tumors, infectious diseases, autoimmune diseases and related diseases and disorders, providing a website featuring medical information, and providing medical information. We plan to register trademarks in connection with our future products.

Government Regulation

Government authorities in the U.S. at the federal, state and local level and in other countries regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of drug and biological products, such as MGTA-456 and any future product candidates. Generally, before a new drug or biologic can be marketed, considerable data demonstrating its quality, safety and efficacy must be obtained, organized into a format specific for each regulatory authority, submitted for review and approved by the regulatory authority.

U.S. drug development

In the U.S., the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations and biologics under the FDCA, the Public Health Service Act, or PHSA, and their

implementing regulations. Both drugs and biologics also are subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or post-market may subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the FDA's refusal to approve pending applications, withdrawal of an approval, a clinical hold, untitled or warning letters, product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

MGTA-456 and any future product candidates must be approved by the FDA through either a New Drug Application, or NDA, or a Biologics License Application, or BLA, process before they may be legally marketed in the U.S. The process generally involves the following:

- completion of extensive preclinical studies in accordance with applicable regulations, including studies conducted in accordance with good laboratory practice, or GLP, requirements;
- submission to the FDA of an application for an investigational new drug, or IND, application, which must become effective before human clinical trials may begin;
- approval by an institutional review board, or IRB, or independent ethics committee at each clinical trial site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, good clinical practice, or GCP, requirements and other clinical trial-related regulations to establish the safety and efficacy of the investigational product for each proposed indication;
- submission to the FDA of an NDA or BLA;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to accept the filing for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities where the drug or biologic will be produced to assess compliance with current good manufacturing processes, or cGMP, requirements to assure that the facilities, methods and controls are adequate to preserve the drug or biologic's identity, strength, quality and purity;
- potential FDA audit of the clinical trial sites that generated the data in support of the NDA or BLA; and
- FDA review and approval of the NDA or BLA, including consideration of the views of any FDA advisory committee, prior to any commercial marketing or sale of the drug or biologic in the U.S.

The preclinical and clinical testing and approval process requires substantial time, effort and financial resources, and we cannot be certain that any approvals for MGTA-456 and any future product candidates will be granted on a timely basis, or at all.

Preclinical studies and IND

Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess safety and, in some cases, to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations for safety/ toxicology studies.

An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, to the

FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before human clinical trials may begin. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time, the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical trials

The clinical stage of development involves the administration of the investigational product to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control, in accordance with GCP requirements, which include the requirement that all research subjects provide their informed consent for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety and assess efficacy. Each protocol, and any subsequent amendments to the protocol, must be submitted to the FDA as part of the IND. Furthermore, each clinical trial must be reviewed and approved by an IRB for each institution at which the clinical trial will be conducted to ensure that the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed. There also are requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries.

A sponsor who wishes to conduct a clinical trial outside of the U.S. may, but often need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor may submit data from the clinical trial to the FDA in support of an NDA or BLA. The FDA will accept a well-designed and well-conducted foreign clinical study not conducted under an IND if the study was conducted in accordance with GCP requirements, and the FDA is able to validate the data through an onsite inspection if deemed necessary.

Clinical trials are generally conducted in three sequential phases, known as Phase 1, Phase 2 and Phase 3, and may overlap.

- Phase 1 clinical trials generally involve a small number of healthy volunteers or disease-affected patients who are initially exposed to a single dose and then multiple doses of the product candidate. The primary purpose of these clinical trials is to assess the metabolism, pharmacologic action, side effect tolerability and safety of the drug.
- Phase 2 clinical trials involve studies in disease-affected patients to determine the dose required to produce the desired benefits. At the same time, safety and further pharmacokinetic and pharmacodynamic information is collected, possible adverse effects and safety risks are identified, and a preliminary evaluation of efficacy is conducted.
- Phase 3 clinical trials generally involve a large number of patients at multiple sites and are designed to provide the data necessary to demonstrate the product candidate's safety and effectiveness for its intended use, and to establish the overall benefit/risk relationship of the product and provide an adequate basis for product labeling.

Post-approval trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA or BLA.

Progress reports detailing the results of the clinical trials, among other information, must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected suspected adverse events, findings from other studies, animal or *in vitro* testing that suggest a significant risk for human subjects and any clinically important increase in the rate of a serious suspected adverse reaction compared to that listed in the protocol or investigator brochure.

Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug or biologic has been associated with unexpected serious harm to patients. Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether a trial may move forward at designated check points based on access to certain data from the trial. Concurrent with clinical trials, companies usually complete additional animal studies, must develop additional information about the chemistry and physical characteristics of the drug or biologic and 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 and, among other things, companies must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidates do not undergo unacceptable deterioration over their shelf life.

NDA/BLA and FDA review process

Following completion of the clinical trials, data are analyzed to assess whether the investigational product is safe and effective for the proposed indicated use or uses. The results of preclinical studies and clinical trials are then submitted to the FDA as part of an NDA or BLA, along with proposed labeling, chemistry and manufacturing information to ensure product quality and other relevant data. The NDA or BLA is a request for approval to market the drug or biologic for one or more specified indications and must contain proof of safety and efficacy for a drug or safety, purity and potency for a biologic. The application may include both negative and ambiguous results of preclinical studies and clinical trials, as well as positive findings. Data may come from company-sponsored clinical trials intended to test the safety and efficacy of a product's use or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational product to the satisfaction of the FDA. FDA approval of an NDA or BLA must be obtained before a drug or biologic may be marketed in the U.S.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each NDA or BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. According to the FDA's fee schedule, effective through September 30, 2019, the user fee for an application requiring clinical data, such as an NDA or BLA, is $2,588,478. The sponsor of an approved NDA or BLA is also subject to an annual prescription drug program fee, which for fiscal year 2019 is $309,915. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs or BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

The FDA reviews all submitted NDAs and BLAs before it accepts them for filing and may request additional information rather than accepting the NDA or BLA for filing. The FDA generally makes a decision on accepting an NDA or BLA for filing within 60 days of receipt. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA or BLA. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has ten months from the filing date to complete its initial review of a new molecular-entity

NDA or original BLA and respond to the applicant, and six months from the filing date of a new molecular-entity NDA or original BLA designated for priority review. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs or BLAs, and the review process is often extended by FDA requests for additional information or clarification.

Before approving an NDA or BLA, the FDA will conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether they comply with cGMP requirements. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications. The FDA also may audit data from clinical trials to ensure compliance with GCP requirements. Additionally, the FDA may refer applications for novel drug products or drug products which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation, and a recommendation as to whether the application should be approved and under what conditions, if any. The FDA is not bound by recommendations of an advisory committee, but it considers such recommendations when making decisions on approval. The FDA likely will reanalyze the clinical trial data, which could result in extensive discussions between the FDA and the applicant during the review process. After the FDA evaluates an NDA or BLA, it will issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug or biologic with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A Complete Response Letter usually describes all of the specific deficiencies in the NDA or BLA identified by the FDA. The Complete Response Letter may require additional clinical data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a Complete Response Letter is issued, the applicant may either resubmit the NDA or BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. Even if such data and information are submitted, the FDA may decide that the NDA or BLA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data.

Orphan drug designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S., or more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making the product available in the U.S. for this type of disease or condition will be recovered from sales of the product.

Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years from the date of such approval, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity by means of greater effectiveness, greater safety, provision of a major contribution to patient care, or in instances of drug supply issues. Competitors, however, may receive approval of either a different product for the same indication or the same product for a different indication but that could be used off-label in the orphan indication. Orphan drug exclusivity could also block the approval of one of our products for seven years if a competitor obtains approval before we do for the same product, as defined by the FDA, for the same indication we are seeking approval, or if our product is determined to be contained within the scope of the competitor’s product for the same indication or disease. If one of our products designated as an

orphan drug receives marketing approval for an indication broader than that which is designated, it may not be entitled to orphan drug exclusivity. Orphan drug status in the European Union has similar, but not identical, requirements and benefits.

Expedited development and review programs

The FDA has a fast track program that is intended to expedite or facilitate the process for reviewing new drugs and biologics that meet certain criteria. Specifically, new drugs and biologics are eligible for fast track designation if they are intended to treat a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast track designation applies to both the product and the specific indication for which it is being studied. The sponsor can request the that FDA designate the product for fast track status any time before receiving NDA or BLA approval, but ideally no later than the pre-NDA or pre-BLA meeting.

Any product submitted to the FDA for marketing, including under a fast track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it treats a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biologic designated for priority review in an effort to facilitate the review.

A product may also be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate 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, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. As a condition of approval, the FDA may require that a sponsor of a drug or biologic receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. If the FDA concludes that a drug or biologic shown to be effective can be safely used only if distribution or use is restricted, it will require such post-marketing restrictions as it deems necessary to assure safe use of the product.

Additionally, a drug or biologic may be eligible for designation as a breakthrough therapy if the product is intended, alone or in combination with one or more other drugs or biologics, to treat a serious or life-threatening condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over currently approved therapies on one or more clinically significant endpoints. The benefits of breakthrough therapy designation include the same benefits as fast track designation plus intensive guidance from the FDA to ensure an efficient drug development program.

Fast track designation, priority review, accelerated approval, and breakthrough therapy designation do not change the standards for approval, but may expedite the development or approval process.

Pediatric information

Under the Pediatric Research Equity Act, or PREA, certain NDAs and BLAs and certain supplements to an NDA or BLA must contain data to assess the safety and efficacy of the drug 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. The FDA may grant deferrals for submission of pediatric data or full or partial waivers. The Food and Drug Administration Safety and Innovation Act, or FDASIA, amended the FDCA to require that a sponsor who is planning to submit a marketing application for a drug that includes a new active ingredient, new indication, new dosage form, new dosing regimen, or new route of administration submit an initial Pediatric Study Plan, or PSP, within 60 days of an end-of-Phase 2 meeting or, if there is no such meeting, as early as practicable before the initiation of the Phase 3 or Phase 2/3 study. The initial PSP must include an outline of the pediatric study or studies that the sponsor plans to conduct, including study objectives and design,

age groups, relevant endpoints and statistical approach, or a justification for not including such detailed information, and any request for a deferral of pediatric assessments or a full or partial waiver of the requirement to provide data from pediatric studies along with supporting information. The FDA and the sponsor must reach an agreement on the PSP. A sponsor can submit amendments to an agreed-upon initial PSP at any time if changes to the pediatric plan need to be considered based on data collected from preclinical studies, early phase clinical trials, and/or other clinical development programs.

Post-marketing requirements

Following approval of a new product, the manufacturer and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and record-keeping activities, reporting of adverse experiences, complying with promotion and advertising requirements, which include restrictions on promoting drugs for unapproved uses or patient populations, or off-label use, and limitations on industry-sponsored scientific and educational activities. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such uses. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Further, if there are any modifications to the drug or biologic, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new NDA/BLA or NDA/BLA supplement, which may require the development of additional data or preclinical studies and clinical trials.

The FDA may also place other conditions on approvals including the requirement for a Risk Evaluation and Mitigation Strategy, or REMS, to assure the safe use of the product. If the FDA concludes a REMS is needed, the sponsor of the NDA or BLA must submit a proposed REMS. The FDA will not approve the NDA or BLA without an approved REMS, if required. A REMS 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. Any of these limitations on approval or marketing could restrict the commercial promotion, distribution, prescription or dispensing of products. Product approvals may be withdrawn for non-compliance with regulatory standards or if problems occur following initial marketing.

FDA regulations require that products be manufactured in specific approved facilities and in accordance with cGMP regulations. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. These manufacturers must comply with cGMP regulations that require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved drugs or biologics 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 requirements and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. The discovery of violative conditions, including failure to conform to cGMP regulations, could result in enforcement actions, and the discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved NDA or BLA, including recall.

Companion diagnostics and complementary diagnostics

We believe that the success of our product candidates may depend, in part, on the development and commercialization of either a companion diagnostic or complementary diagnostic. Companion diagnostics and complementary diagnostics can identify patients who are most likely to benefit from a particular therapeutic product; identify patients likely to be at increased risk for serious side effects as a result of treatment with a particular therapeutic product; or monitor response to treatment with a particular therapeutic product for the purpose of adjusting treatment to achieve improved safety or effectiveness. Companion diagnostics and complementary diagnostics are regulated as medical devices by the FDA and, as such, require either clearance or

approval prior to commercialization. The level of risk combined with available controls to mitigate risk determines whether a companion diagnostic device requires Premarket Approval Application, or PMA, approval or is cleared through the 510(k) premarket notification process. For a novel therapeutic product for which a companion diagnostic device is essential for the safe and effective use of the product, the companion diagnostic device should be developed and approved or 510(k)-cleared contemporaneously with the therapeutic. The use of the companion diagnostic device will be stipulated in the labeling of the therapeutic product. This is also true for a complementary diagnostic, although it is not a prerequisite for receiving the therapeutic.

Other regulatory matters

Manufacturing, sales, promotion and other activities following product approval are also subject to regulation by numerous regulatory authorities in the U.S. in addition to the FDA, including the Centers for Medicare & Medicaid Services, or CMS, other divisions of the Department of Health and Human Services, the Department of Justice, the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency and state and local governments.

Healthcare providers, physicians, and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third party payors, healthcare providers and physicians 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 any drugs for which we obtain marketing approval. In the U.S., these laws include: the federal Anti-Kickback Statute, the False Claims Act, and the Health Insurance Portability and Accountability Act of 1996, or HIPAA.

The Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer (or a party acting on its behalf), to knowingly and willfully solicit, receive, offer or pay any remuneration, directly or indirectly, in cash or in kind, that is intended to induce or reward referrals, including the purchase, recommendation, order or prescription of a particular drug, for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties and exclusion from participation in federal healthcare programs. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it.

The federal False Claims Act imposes civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities (including manufacturers) 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. Penalties for a False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties of between $11,181 and $22,363 for each separate false claim, the potential for exclusion from participation in federal healthcare programs, and the potential implication of various federal criminal statutes. The government may deem manufacturers to have "caused" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. Claims which include items or services resulting from a violation of the federal Anti-Kickback Statute are false or fraudulent claims for purposes of the False Claims Act. Our future marketing and activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, as well as the sale and marketing of our product and any future product candidates, are subject to scrutiny under this law.

HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters, and, as amended by the Health Information

Technology for Economic and Clinical Health Act of 2009, or HITECH, and their implementing regulations, also imposes obligations, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information held by covered entities and their business associates. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;

The Physician Payments Sunshine Act of 2010, which requires applicable manufacturers of covered drugs (those paid for by a federal healthcare program) to report to CMS any payments and other transfers of value made to physicians and teaching hospitals.

Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services. Such laws are generally broad and are enforced by various state agencies and private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant federal government compliance guidance and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare 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 pre-empted by HIPAA, thus complicating compliance efforts.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of drugs from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Ensuring business arrangements comply with applicable healthcare laws, as well as responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention away from the business.

Current and Future Legislation

In the U.S. and foreign jurisdictions, there have been 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 to profitably sell any product candidates for which we 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 in additional downward pressure on the price that we, or any collaborators, may receive for any approved products.

With the new administration and Congress, there will likely be additional administrative or legislative changes, including modification, repeal, or replacement of all, or certain provisions of, the Affordable Care Act, or ACA, which may impact reimbursement for drugs and biologics. For example, in Congress, the U.S. House of Representatives passed ACA replacement legislation known as the American Health Care Act of 2017 in May 2017, which was not introduced in the Senate. However, The Tax Cuts and Jobs Act of 2017, or TCJA, includes

a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. The current administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal. Congress may consider other legislation to repeal or replace certain elements of the ACA. In addition, since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Further, the Trump administration has concluded that cost-sharing reduction, or CSR, payments to insurance companies required under the ACA have not received necessary appropriations from Congress and announced that it will discontinue these payments immediately until such appropriations are made. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the ACA. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain. Several state attorneys general filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group market places, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Packaging and Distribution in the U.S.

If our products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The failure to comply with any of these laws or regulatory requirements subjects firms to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, exclusion from federal healthcare programs, requests for recall, seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a firm to enter into supply contracts, including government contracts. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Prohibitions or restrictions on sales or withdrawal of future products marketed by us could materially affect our business in an adverse way.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

Other U.S. environmental, health and safety laws and regulations

We may be 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. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our 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 for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

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

U.S. patent term restoration and marketing exclusivity

Depending upon the timing, duration and specifics of FDA approval of MGTA-456 and any future product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit restoration of the patent term of up to five years as compensation for patent term lost during product development and FDA regulatory review process. Patent-term restoration, however, cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent-term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA or BLA plus the time between the submission date of an NDA or BLA and the approval of that application, except that the review period is reduced by any time during which the applicant failed to exercise due diligence. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. PTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA or BLA.

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the U.S. to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

An abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product was created by the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, as part of the ACA. This amendment to the PHSA, in part, attempts to minimize duplicative testing. Biosimilarity, which requires that the biological product be highly similar to the reference product notwithstanding minor differences in clinically inactive components and that there be no clinically meaningful differences between the product and the reference product in terms of safety, purity and potency, can be shown through analytical studies, animal studies and a clinical trial or trials. Interchangeability requires that a biological product be biosimilar to the reference product and that the product can be expected to produce the same clinical results as the reference product in any given patient and, for products administered multiple times to an individual, that the product and the reference product may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biological product without such alternation or switch. Complexities associated with the larger, and often more complex, structure of biological products as compared to small molecule drugs, as well as the processes by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biological product is granted four- and twelve-year exclusivity periods from the time of first licensure of the product. The FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product, and the FDA will not approve an application for a biosimilar or interchangeable product based on the reference biological product until twelve years after the date of first licensure of the reference product. "First licensure" typically means the initial date the particular product at issue was licensed in the U.S. Date of first licensure does not include the date of licensure of (and a new period of exclusivity is not available for) a biological product if the licensure is for a supplement for the biological product or for a subsequent application by the same sponsor or manufacturer of the biological product (or licensor, predecessor in interest, or other related entity) for a change (not including a modification to the structure of the biological product) that results in a new indication, route of administration, dosing schedule, dosage form, delivery system, delivery device or strength, or for a modification to the structure of the biological product that does not result in a change in safety, purity, or potency. Therefore, one must determine whether a new product includes a modification to the structure of a previously licensed product that results in a change in safety, purity, or potency to assess whether the licensure of the new product is a first licensure that triggers its own period of exclusivity. Whether a subsequent application, if approved, warrants exclusivity as the "first licensure" of a biological product is determined on a case-by-case basis with data submitted by the sponsor.

Pediatric exclusivity is another type of regulatory market exclusivity in the U.S. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods. This six-month exclusivity may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "Written Request" for such a trial.

European Union drug development

In the European Union, our future products also may be subject to extensive regulatory requirements. As in the U.S., medicinal products can be marketed only if a marketing authorization from the competent regulatory agencies has been obtained.

Similar to the U.S., the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC has sought to harmonize the EU clinical trials regulatory framework, setting out common rules for the control and authorization of clinical trials in the EU, the EU Member States have transposed and applied the provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved in each of the EU countries where the trial is to be conducted by two distinct bodies: the National Competent Authority, or NCA, and one or more Ethics Committees, or ECs. Under the

current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial have to be reported to the NCA and ECs of the Member State where they occurred.

The EU clinical trials legislation currently is undergoing a transition process mainly aimed at harmonizing and streamlining clinical-trial authorization, simplifying adverse-event reporting procedures, improving the supervision of clinical trials and increasing their transparency. Recently enacted Clinical Trials Regulation EU No. 536/2014 ensures that the rules for conducting clinical trials in the EU will be identical.

European Union drug marketing

Much like the Anti-Kickback Statue prohibition in the U.S., the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

European Union drug review and approval

In the European Economic Area, or EEA, which is comprised of the 27 Member States of the European Union (including Norway and excluding Croatia), Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of marketing authorizations.

- The Community MA is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use, or CHMP, of the European Medicines Agency, or EMA, and is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, advanced-therapy medicines such as gene-therapy, somatic cell-therapy or tissue-engineered medicines and medicinal products containing a new active substance indicated for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU.
- National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in another Member State through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent authorities of each of the Member States in which the MA is sought, one of which is selected by the applicant as the Reference Member State, or RMS. The competent authority of the RMS prepares a draft assessment report, a draft summary of the product characteristics, or SPC, and a draft of the labeling and package leaflet, which are sent to the other Member States (referred to as the Member States Concerned) for their approval. If the Member States Concerned raise no objections, based on a

potential serious risk to public health, to the assessment, SPC, labeling, or packaging proposed by the RMS, the product is subsequently granted a national MA in all the Member States (i.e., in the RMS and the Member States Concerned).

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

European Union new chemical entity exclusivity

In the European Union, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. The data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten 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 determined to bring a significant clinical benefit in comparison with currently approved therapies.

European Union orphan designation and exclusivity

In the European Union, the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union community (or where it is unlikely that the development of the medicine would generate sufficient return to justify the investment) and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or, if a method exists, the product would be a significant benefit to those affected).

In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

European Data Collection

The collection and use of personal health data in the European Union is governed by the provisions of the Data Protection Directive, and as of May 2018 the General Data Protection Regulation, or GDPR. This directive imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive and GDPR also impose strict rules on the transfer of personal data out of the European Union to the U.S. Failure to comply with the requirements of the Data Protection Directive, the GDPR, and the related national data protection laws of the European Union Member States may result in fines and other administrative penalties. The GDPR introduces new data protection requirements in the European Union and substantial fines for breaches of the data protection rules. Notably, on January 21, 2019, Google was fined almost $57 million by French regulators for violating GDPR. The GDPR regulations may impose additional responsibility and liability in relation to personal data that we process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and adversely affect our business, financial condition, results of operations and prospects.

Rest of the world regulation

For other countries outside of the European Union and the U.S., such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Additionally, the clinical trials must be conducted in accordance with GCP requirements and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Additional laws and regulations governing international operations

If we further expand our operations outside of the U.S., we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or 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 U.S. 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.

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

Various laws, regulations and executive orders also restrict the use and dissemination outside of the U.S., 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 U.S., 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 products and product candidates outside of the U.S., 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 civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Reimbursement

Sales of our products will depend, in part, on the extent to which our products will be covered by third-party payors, such as government health programs, commercial insurance and managed healthcare organizations. In the U.S., no uniform policy of coverage and reimbursement for drug or biological products exists. Accordingly, decisions regarding the extent of coverage and amount of reimbursement to be provided for any of our products will be made on a payor-by-payor basis. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including

price-controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. For example, the ACA contains provisions that subject biological products to potential competition by lower-cost biosimilars and may reduce the profitability of drug products through increased rebates for drugs reimbursed by Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Adoption of general controls and measures, coupled with the tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceutical drugs.

The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. The ACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers' rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs from 15.1% of average manufacturer price, or AMP, to 23.1% of AMP and adding a new rebate calculation for "line extensions" (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, creating a new methodology by which rebates owed are calculated for drugs that are inhaled, infused, instilled, implanted or injected, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The ACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization and by enlarging the population potentially eligible for Medicaid drug benefits. Pricing and rebate programs must also comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Unlike Medicare Part A and B, Part D coverage is not standardized. While all Medicare drug plans must give at least a standard level of coverage set by Medicare, Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval. However, any negotiated prices for our products covered by a Part D prescription drug plan likely will be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

For a drug product to receive federal reimbursement under the Medicaid or Medicare Part B programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. As of 2010, the ACA expanded the types of entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs. In addition, as 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

As noted above, the marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement.

An increasing emphasis on cost containment measures in the U.S. has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

In recent years, additional laws have resulted in direct or indirect reimbursement reductions for certain Medicare providers.

- 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. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2027 unless additional Congressional action is taken.
- The American Taxpayer Relief Act of 2012, 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.
- The Middle Class Tax Relief and Job Creation Act of 2012 required that the Centers for Medicare & Medicaid Services reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting.

In addition, the current administration released a "Blueprint," or plan, to reduce the cost of drugs. The Trump administration's Blueprint contains certain measures that the U.S. Department of Health and Human Services is already working to implement. For example, on October 25, 2018, CMS issued an Advanced Notice of Proposed Rulemaking, or ANPRM, indicating it is considering issuing a proposed rule in the spring of 2019 on a model called the International Pricing Index, or IPI. This model would utilize a basket of other countries' prices as a reference for the Medicare program to use in reimbursing for drugs covered under Part B. The ANPRM also included an updated version of the Competitive Acquisition Program as an alternative to current "buy and bill" payment methods for Part B drugs. Individual state legislatures have become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing. Some of these measures include price or patient reimbursement constraints, discounts, restrictions on certain product access, marketing cost disclosure and transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which 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.

In addition, in most foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various European Union member states

and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. A member state may approve a specific price for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the U.S. and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of any of our product candidates in those countries would be negatively affected.

Employees

As of December 31, 2018, we had 59 full-time employees, 25 of our employees have Ph.D. or M.D. degrees and 40 of our employees are engaged in research and development activities.

Our Corporate Information

We were incorporated under the laws of the State of Delaware on June 17, 2015 under the name HSCTCo Therapeutics, Inc. In February 2016, we changed our name to Magenta Therapeutics, Inc.

On June 25, 2018, we completed the initial public offering ("IPO") pursuant to which we issued and sold 6,666,667 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of $89.9 million, after deducting underwriting discounts and commissions and other offering expenses. Upon the closing of the IPO, our outstanding redeemable convertible preferred stock automatically converted into shares of common stock.

See Part II—Item 7—Management's Discussion and Analysis of Financial Condition and Results of Operations and Note 1 to the consolidated financial statements included in Part II—Item 8 for more information about the above-mentioned transactions.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of: (i) the last day of the fiscal year (a) following the fifth anniversary of the completion of the IPO, (b) in which we have total annual gross revenue of at least $1.07 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, and (ii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Our principal executive offices are located at 100 Technology Square, Cambridge, MA 02139, and our telephone number is (857) 242-0170. Our website address is *www.magentatx.com*. Our website and the information contained on, or that can be accessed through, the website will not be deemed to be incorporated by reference in, and are not considered part of, this Annual Report on Form 10-K.

Available Information

Our Internet address is *www.magentatx.com*. Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, including exhibits, proxy and information statements and amendments to those reports filed or furnished pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, are available through the "Investors" portion of our website free of charge as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. Information on our website is not part of this Annual Report on Form 10-K or any of our other securities filings unless specifically incorporated herein by reference. In addition, our filings with the SEC may be accessed through the SEC's Interactive Data Electronic Applications system at *http://www.sec.gov*. All statements made in any of our securities filings, including all forward-looking statements or information, are made as of the date of the document in which the statement is included, and we do not assume or undertake any obligation to update any of those statements or documents unless we are required to do so by law.

ITEM 1A. RISK FACTORS

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Annual Report on Form 10-K and in other documents that we file with the Securities and Exchange Commission, or SEC, in evaluating the Company and our business. Investing in our common stock involves a high degree of risk. If any of the following risks and uncertainties actually occurs, our business, prospects, financial condition and results of operations could be materially and adversely affected. The risks described below are not intended to be exhaustive and are not the only risks facing the Company. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impact our business, prospects, financial condition and results of operations.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.

We are a clinical-stage biotechnology company developing novel medicines to bring the curative power of stem cell transplant to more patients and have a limited operating history. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale and have not generated any revenue from product sales to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in each period since our inception in June 2015. For the years ended December 31, 2018, 2017 and 2016, we reported a net loss of $57.5 million, $35.5 million and $9.4 million, respectively. As of December 31, 2018, we had an accumulated deficit of $102.7 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development costs and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We will require additional capital to fund our operations and if we fail to obtain necessary financing, we will not be able to complete the development and commercialization of our product candidates.

Our operations have consumed substantial amounts of cash since our inception. We expect to continue to spend substantial amounts of cash (including the net proceeds from our initial public offering, or IPO) to conduct further research and development and preclinical testing and clinical trials of our product candidates, to seek regulatory approvals for our product candidates and to launch and commercialize any product candidates for which we receive regulatory approval, including potentially building our own commercial organization to address the United States, the European Union and certain other markets. As of December 31, 2018, we had approximately $142.6 million in cash, cash equivalents and marketable securities. Our monthly spending levels will vary based on new and ongoing development and corporate activities. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our product candidates;

- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates that we develop or may in-license;
- the terms of any collaboration agreements we may choose to conclude;
- the outcome, timing and cost of meeting regulatory requirements established by the FDA, the European Medical Agency, or EMA, and other comparable foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the effect of competing technological and market developments;
- the cost and timing of completion of commercial-scale outsourced manufacturing activities; and
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own.

We cannot be certain that additional funding will be available on acceptable terms, or at all. 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 one or more of our product candidates or one or more of our other research and development initiatives. We also could be required to seek collaborators for one or more of our current or future 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 technologies or product candidates that we otherwise would seek to develop or commercialize 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 existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholder's ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect our stockholder's rights. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates or grant licenses on terms unfavorable to us.

Our company has a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

We are an early-stage company. We were founded and commenced operations in June 2015. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates, and undertaking preclinical studies, and in the case of MGTA-456, clinical trials. Aside from MGTA-456, all of our research programs and product

candidates are still in the preclinical or research stage of development, and their risk of failure is high. We have not yet demonstrated an ability to initiate or successfully complete any clinical trials (other than for MGTA-456), including large-scale, pivotal clinical trials; obtain marketing approvals; manufacture a commercial-scale medicine, or arrange for a third party to do so on our behalf; or conduct sales and marketing activities necessary for successful commercialization. Typically, it takes about 10 to 15 years to develop a new medicine from the time it is discovered to when it is available for treating patients. Consequently, any predictions we make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays, and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We have never generated revenue from product sales and may never be profitable.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize product candidates we may identify for development. We may not generate revenues from product sales for the next several years, if ever. Our ability to generate future revenues from product sales depends heavily on our, or our collaborators', ability to successfully:

- identify product candidates and complete research and preclinical and clinical development of any product candidates we may identify;
- seek and obtain regulatory and marketing approvals for any of our product candidates for which we complete clinical trials;
- launch and commercialize any of our product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing, and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualify for adequate coverage and reimbursement by government and third-party payors for any of our product candidates for which we obtain regulatory and marketing approval;
- develop, maintain, and enhance a sustainable, scalable, reproducible, and transferable manufacturing process for the product candidates we may develop;
- establish and maintain supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for any of our product candidates for which we obtain regulatory and marketing approval;
- obtain market acceptance of any product candidates we may develop as viable treatment options;
- address competing technological and market developments;
- implement internal systems and infrastructure, as needed;
- negotiate favorable terms in any collaboration, licensing, or other arrangements into which we may enter and perform our obligations in such collaborations;
- maintain, protect, and expand our portfolio of intellectual property rights, including patents, trade secrets, and know-how;
- avoid and defend against third-party interference or infringement claims; and
- attract, hire, and retain qualified personnel.

Even if one or more of the product candidates we may develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our

expenses could increase beyond expectations if we are required by the FDA, the EMA, or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Comprehensive tax reform legislation could adversely affect our business and financial condition.

The U.S. government has recently enacted comprehensive tax legislation that includes significant changes to the taxation of business entities. These changes include, among others, a permanent reduction to the corporate income tax rate. Notwithstanding the reduction in the corporate income tax rate, the overall impact of this tax reform is uncertain, and our business and financial condition could be adversely affected. This Annual Report on Form 10-K does not discuss any such tax legislation or the manner in which it might affect purchasers of our common stock. We urge our stockholders to consult with their legal and tax advisors with respect to any such legislation and the potential tax consequences of investing in our common stock.

Our insurance policies may be inadequate and potentially expose us to unrecoverable risks.

We have limited director and officer insurance and commercial insurance policies. Any significant insurance claims would have a material adverse effect on our business, financial condition and results of operations. Insurance availability, coverage terms and pricing continue to vary with market conditions. We endeavor to obtain appropriate insurance coverage for insurable risks that we identify; however, we may fail to correctly anticipate or quantify insurable risks, we may not be able to obtain appropriate insurance coverage, and insurers may not respond as we intend to cover insurable events that may occur. We have observed rapidly changing conditions in the insurance markets relating to nearly all areas of traditional corporate insurance. Such conditions have resulted in higher premium costs, higher policy deductibles and lower coverage limits. For some risks, we may not have or maintain insurance coverage because of cost or availability.

Risks Related to Product Development and Regulatory Approval

We are very early in our development efforts. All but one of our product candidates, MGTA-456, are still in preclinical development. If we are unable to advance our product candidates to obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and all but one of our product candidates, MGTA-456, are still in preclinical development. We have only recently completed initial preclinical studies for MGTA-145. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

Each of our programs and product candidates will require additional preclinical and clinical development; regulatory approval in multiple jurisdictions; obtaining manufacturing supply, capacity and expertise; building of a commercial organization; substantial investment and significant marketing efforts before we generate any revenue from product sales. Our product candidates must be authorized for marketing by the FDA, or certain other foreign regulatory agencies, such as the EMA, before we may commercialize our product candidates.

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

- successful completion of preclinical studies and successful enrollment and completion of clinical trials, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, under the FDA's current Good Clinical Practices, or cGCPs, and the FDA's current Good Laboratory Practices, or cGLPs;

- effective Investigational New Drug applications, or INDs, or Clinical Trial Authorisations, or CTAs, that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- positive results from our future clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile of our product candidates in the intended populations;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishment of arrangements with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- successful development of our internal manufacturing processes or transfer to larger-scale facilities operated by either a contract manufacturing organization, or CMO, or by us;
- establishment and maintenance of patent and trade secret protection or regulatory exclusivity for our product candidates;
- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;
- effective competition with other therapies;
- establishment and maintenance of healthcare coverage and adequate reimbursement;
- enforcement and defense of intellectual property rights and claims; and
- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

Our planned clinical trials or those of our collaborators may reveal significant adverse events not seen in our preclinical studies and may result in a safety profile that could inhibit regulatory approval or market acceptance of any of our product candidates.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. For example, we have observed a limited number of serious adverse events in our Phase 2 clinical trial of MGTA-456 in blood cancers that were considered to be related to the investigational treatment, and there is no guarantee that we will not see more serious adverse events in the future. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier trials. Most product candidates that commence clinical trials are never approved as products.

If significant adverse events or other side effects are observed in any of our current or future clinical trials, we may have difficulty recruiting patients to the clinical trial, patients may drop out of our trial, or we may be

required to abandon the trial or our development efforts of that product candidate altogether. We, the FDA or other applicable regulatory authorities, or an institutional review board, or IRB, may suspend clinical trials of a product candidate at any time for various reasons, including a belief that subjects in such trials are being exposed to unacceptable health risks or adverse side effects. Some potential therapeutics developed in the biotechnology industry that initially showed therapeutic promise in early-stage studies have later been found to cause side effects that prevented their further development. Even if the side effects do not preclude the drug from obtaining or maintaining marketing approval, undesirable side effects may inhibit market acceptance of the approved product due to its tolerability versus other therapies. Any of these developments could materially harm our business, financial condition and prospects.

Clinical development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of any product candidates.

With the exception of MGTA-456, our other product candidates are still in the preclinical development stage, and their risk of failure is high. It is impossible to predict when or if any of our programs will prove effective and safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of any of our future product candidates in humans. Preclinical and clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. We may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, and a clinical trial can fail at any stage of testing. The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

Successful completion of clinical trials is a prerequisite to submitting a new drug application, or NDA, or a biologics license application, or BLA, to the FDA, a Marketing Authorization Application, or MAA, to the EMA and similar approval filings to comparable foreign regulatory authorities, for each product candidate and, consequently, the ultimate approval and commercial marketing of any product candidates. We do not know whether any of our clinical trials will begin or be completed on schedule, if at all.

We may experience delays in completing our preclinical studies and initiating or completing clinical trials. We also may experience numerous unforeseen events during, or as a result of, any future clinical trials that we could conduct that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- regulators, IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable terms with prospective trial sites and prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of any product candidates may fail to show safety or efficacy, produce negative or inconclusive results and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials or we may decide to abandon product development programs;
- the number of patients required for clinical trials of any product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;

- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or may deviate from the clinical trial protocol or drop out of the trial, which may require that we add new clinical trial sites or investigators;
- we may elect to, or regulators, IRBs or ethics committees may require that we or our investigators, suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of preclinical studies and clinical trials of any product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, IRBs or ethics committees to suspend or terminate the trials, or reports may arise from preclinical or clinical testing of other stem cell transplant and cell-based therapies that raise safety or efficacy concerns about our product candidates.

We could also encounter delays if a clinical trial is suspended or terminated by us, the IRBs of the institutions in which such trials are being conducted, the Data Safety Monitoring Board, or DSMB, for such trial or FDA or other regulatory authorities. Such authorities may impose such a suspension or termination 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 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 or treatment, failure to establish or achieve clinically meaningful trial endpoints, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. For example, in December 2015, prior to our license of MGTA-456 from Novartis International Pharmaceutical Ltd., or Novartis, the FDA imposed a partial clinical hold on the cryopreserved part of the protocol covered by the IND application for MGTA-456 until Novartis demonstrated comparability between the fresh and cryopreserved product. This partial clinical hold was later removed by the FDA in June 2016 after Novartis presented satisfactory comparability data between the fresh and cryopreserved product. We cannot guarantee that we will not be subject to further holds by the FDA or other regulatory authorities in the future. In addition, 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. Further, the FDA or other regulatory authorities may disagree with our clinical trial design and our interpretation of data from clinical trials or may change the requirements for approval even after they have reviewed and commented on the design for our clinical trials.

Our product development costs will increase if we experience delays in clinical testing or marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates and may allow our competitors to bring products to market before we do, potentially impairing our ability to successfully commercialize our product candidates and harming our business and results of operations. Any delays in our preclinical or future clinical development programs may harm our business, financial condition and prospects significantly.

We have no experience as a company in obtaining regulatory approval for a drug.

As a company, we have never obtained regulatory approval for, or commercialized, a drug. It is possible that the FDA may refuse to accept any or all future NDA or BLAs for substantive review or may conclude after review of our data that our application is insufficient to obtain regulatory approval for any current or future

product candidates. If the FDA does not approve any future NDAs or BLAs, it may require that we conduct additional costly clinical, preclinical or manufacturing validation studies before it will reconsider our applications. Depending on the extent of these or any other FDA-required studies, approval of any NDA or BLA or other application that we submit may be significantly delayed, possibly for several years, or may require us to expend more resources than we have available. Any failure or delay in obtaining regulatory approvals would prevent us from commercializing MGTA-456 or any other product candidate, generating revenues and achieving and sustaining profitability. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the FDA to approve any NDA or other application that we submit. If any of these outcomes occur, we may be forced to abandon the development of our product candidates, which would materially adversely affect our business and could potentially cause us to cease operations. We face similar risks for our applications in foreign jurisdictions.

If serious adverse events, undesirable side effects, or unexpected characteristics are identified during the development of any product candidates we may develop, we may need to abandon or limit our further clinical development of those product candidates.

It is impossible to predict when or if any product candidates we may develop will prove safe in humans. If any product candidates we develop are associated with serious adverse events, or undesirable side effects, or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe, or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects. It is possible that product candidates that initially showed promise in early stage testing will later have been found to cause side effects that prevent further clinical development of the product candidates.

Stem cell transplant is a high-risk procedure with curative potential that may result in complications or adverse events for patients in our clinical trials or for patients that use any of our product candidates, if approved.

Stem cell transplant can cure patients across multiple diseases, but its use carries with it risks of toxicity, serious adverse events and death. Because many of our therapies are used to prepare or treat patients undergoing stem cell transplant, patients in our clinical trials or patients that use any of our product candidates may be subject to many of the risks that are currently inherent to the stem cell transplant process. In particular, stem cell transplant involves certain known potential post-procedure complications that may manifest several weeks or months after a transplant and which may be more common in certain patient populations. For example, up to 20% of patients with inherited metabolic diseases treated with a transplant experience primary engraftment failure, resulting in severe complications, including death. We have reported that patients treated with MGTA-456 in our ongoing Phase 2 study successfully engrafted, and subsequently developed autoimmune cytopenia, a known and frequent complication of the transplant procedure. The first patient in our Phase 2 study in inherited metabolic disorders subsequently died from this complication. These cytopenias were deemed by both the principal investigator overseeing the Phase 2 study and the chair of our data safety monitoring committee to be unrelated to the use of MGTA-456 specifically but instead were deemed to be related to complications common to the transplant procedure. If these or other serious adverse events, undesirable side effects, or unexpected characteristics are identified during the development of any of our product candidates, we may need to limit, delay or abandon our further clinical development of those product candidates, even if such events, effects or characteristics were the result of stem cell transplant or related procedures generally, and not directly or specifically caused or exacerbated by our product candidates. All serious adverse events or unexpected side effects are continually monitored per the clinical study's approved protocol. If serious adverse events are determined to be directly or specifically caused or exacerbated by our product candidates, we would follow the study protocol's requirements, which call for our data safety monitoring committee to review all available clinical data in making a recommendation regarding the study's continuation.

If we are not able to identify a safe and effective dose for any of our ADCs, we may need to delay, abandon or limit our development of any potential product candidates.

ADCs utilize toxins to kill cells, and we may not be able to identify a safe and effective dose for some of our potential product candidates. ADCs, including those that have received marketing approval, have dose-dependent safety findings that can include liver toxicity, depending on the target of the ADC and the drug used in the conjugate. In addition, ADCs may have other adverse side effects including fatalities. Although our probe CD117-ADC which was designed to deplete hematopoietic stem cells, or HSCs, was generally well tolerated at efficacious doses in non-human primate studies, we may not be able to ultimately show that an optimized CD117-ADC can deplete HSCs at a safe and effective dose in humans and we may need to delay, abandon or limit these development efforts.

Even if we obtain regulatory approval of any of our product candidates, the approved products may be subject to post-approval studies and will remain subject to ongoing regulatory requirements. If we fail to comply, or if concerns are identified in subsequent studies, our approval could be withdrawn, and our product sales could be suspended.

If we are successful at obtaining regulatory approval for MGTA-456 or any of our other product candidates, regulatory agencies in the United States and other countries where a product will be sold may require extensive additional clinical trials or post-approval clinical trials that are expensive and time-consuming to conduct. In particular, therapeutic products administered for the treatment of certain inherited metabolic diseases, such as Hurler syndrome and leukodystrophies, are likely to require extensive follow-up studies and close monitoring of patients after regulatory approval has been granted, for any signs of adverse effects that occur over a long period of time. These studies may be expensive and time-consuming to conduct and may reveal side effects or other harmful effects in patients that use our therapeutic products after they are on the market, which may result in the limitation or withdrawal of our drugs from the market. Alternatively, we may not be able to conduct such additional trials, which might force us to abandon our efforts to develop or commercialize certain product candidates. Even if post-approval studies are not requested or required, after our products are approved and on the market, there might be safety issues that emerge over time that require a change in product labeling, additional postmarket studies or clinical trials, imposition of distribution and use restrictions under a Risk Evaluation and Mitigation Strategy, or REMS, or withdrawal of the product from the market, which would cause our revenue to decline.

Additionally, any products that we may successfully develop will be subject to ongoing regulatory requirements after they are approved. These requirements will govern the manufacturing, packaging, marketing, distribution, and use of our products. If we fail to comply with such regulatory requirements, approval for our products may be withdrawn, and product sales may be suspended. We may not be able to regain compliance, or we may only be able to regain compliance after a lengthy delay, significant expense, lost revenues and damage to our reputation.

Because we are developing product candidates for the treatment of diseases in which there is little clinical experience using new technologies, there is increased risk that the FDA, the EMA, or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

During the regulatory review process, we will need to identify success criteria and endpoints such that the FDA, the EMA, or other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop. As we are initially seeking to identify and develop product candidates to treat diseases in which there is little clinical experience using new technologies, there is heightened risk that the FDA, the EMA, or other regulatory authorities may not consider the clinical trial endpoints that we propose to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently

validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. This may be a particularly significant risk for many of the genetically defined diseases for which we plan to develop product candidates because many of these diseases have small patient populations and designing and executing a rigorous clinical trial with appropriate statistical power is more difficult than with diseases that have larger patient populations. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the European Union and other countries, such as the Committee for Advanced Therapies, or CAT, may make similar comments with respect to these endpoints and data. Any product candidates we may develop will be based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval.

A Breakthrough Therapy Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We plan to seek a Breakthrough Therapy Designation for our product candidates if the clinical data support such a designation for one or more product candidates. A breakthrough therapy is defined as a drug or biologic that 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 drug, or biologic, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Biologics designated as breakthrough therapies by the FDA may also be eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe 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. In any event, the receipt of a Breakthrough Therapy Designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under non-expedited FDA review 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 no longer meets the conditions for qualification.

Inadequate funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

For example, over the last several years, including beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough

critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Our current product candidates and any future product candidates may not be eligible for Orphan Drug status.

The United States and Europe may designate drugs for relatively small patient populations as orphan drugs. Orphan Drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process, but does make the product eligible for orphan drug exclusivity, reduced filing fees and specific tax credits. Generally, if a company receives the first marketing approval for a product with an Orphan Drug designation in the clinical indication for which it has such designation, the product is entitled to orphan drug exclusivity. Orphan drug exclusivity means that the FDA will not approve another application to market the same drug for the same indication, except in limited circumstances, for a period of seven years in the United States. This exclusivity, however, could block the approval of our proposed product candidates if a competitor obtains marketing approval before us. However, even if we obtain orphan drug exclusivity for any of our proposed product candidates, we may not be able to maintain it. For example, if a competitive product is shown to be clinically superior to our product candidates, any orphan drug exclusivity we have will not block the approval of such competitive product.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. 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 study until its conclusion. The enrollment of patients depends on many factors, including:

- the patient eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoints;
- the proximity of patients to study sites;
- the design of the trial;
- our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- our ability to obtain and maintain patient consents; and
- the risk that patients enrolled in clinical trials will drop out of the trials before completion.

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. Since the number of qualified clinical investigators is limited, 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 in such clinical trial site.

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.

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

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial medicines or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. 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 collaboration, 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. Any such event could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate we may develop, and any such approval may be for a more narrow indication than we seek.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials, and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or distribution and use restrictions under a REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use, or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. Any of the foregoing scenarios could materially harm the commercial prospects for any product candidates we may develop and materially adversely affect our business, financial condition, results of operations, and prospects.

A Fast Track Designation by the FDA, even if granted for any of our product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive marketing approval.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA Fast Track Designation for a particular indication. Marketing applications filed by sponsors of products in Fast Track development may qualify for priority review under the policies and procedures offered by the FDA, but the Fast Track Designation does not assure any such qualification or ultimate marketing approval by the FDA. Receipt of Fast Track Designation may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures. In addition, the FDA may withdraw any Fast Track Designation at any time. We may seek Fast Track Designation for MGTA-456 or any other product candidates, but there is no assurance that the FDA will grant this status to any of our proposed product candidates.

We may seek priority review designation for MGTA-456 or our other product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates, however, we cannot assume that MGTA-456 or our other product candidates will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

Results of preclinical studies and early clinical trials may not be predictive of results of future clinical trials, and such results do not guarantee approval of a product candidate by regulatory authorities.

The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of clinical trials do not necessarily predict success in the results of completed clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we could face similar setbacks. The design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain regulatory approval for their product candidates. Even if we complete clinical development of MGTA-456, there can be no assurance that the FDA, EMA, or other regulatory authorities will approve MGTA-456 for marketing.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial procedures and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidate, and, correspondingly, our business and financial prospects would be negatively impacted.

Our product candidates for which we intend to seek approval may face competition from generic drugs or biosimilars sooner than anticipated.

With the enactment of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, an abbreviated pathway for the approval of biosimilar and interchangeable biological products was created. The abbreviated regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as interchangeable based on its similarity to an existing reference product. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original branded product is approved under a BLA. On March 6, 2015, the FDA approved the first biosimilar product under the BPCIA. However, the law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that if any of our product candidates are approved as a biological product under a BLA it should qualify for the 12-year period of exclusivity. However, there is a risk that the FDA will not consider any of our product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Additionally, this period of regulatory exclusivity does not apply to companies pursuing regulatory approval via their own traditional BLA, rather than via the abbreviated pathway. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. Finally, there has been public discussion of potentially decreasing the period of exclusivity from the current 12 years. If such a change were to be enacted, our product candidates, if approved, could have a shorter period of exclusivity than anticipated.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products for which we obtain marketing approval.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the Affordable Care Act, or the ACA, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. As implementation of the ACA is ongoing, the law appears likely to continue the downward pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

Moreover, the Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

As an early stage small company that will be competing against numerous large, established companies that have substantially greater financial, technical, research, manufacturing, marketing, distribution and other resources than us, we will be at a significant competitive disadvantage.

The pharmaceutical and biopharmaceutical industry is characterized by intense competition and rapid and significant technological changes and advancements. Many companies, research institutions and universities are doing research and development work in a number of areas similar to those that we focus on that could lead to the development of new products which could compete with and be superior to our product candidates.

Most of the companies with which we compete have substantially greater financial, technical, research, manufacturing, marketing, distribution and other resources than those of ours. A number of these companies may have or may develop technologies for developing products for treating various diseases, including certain inherited metabolic diseases such as Hurler syndrome and leukodystrophies, that could prove to be superior to ours. We expect technological developments in the pharmaceutical and biopharmaceutical and related fields to occur at a rapid rate, and we believe competition will intensify as advances in these fields are made. Accordingly, we will be required to continue to devote substantial resources and efforts to research and development activities

in order to potentially achieve and maintain a competitive position in this field. Products that we develop may become obsolete before we are able to market them or to recover all or any portion of our research and development expenses. We will be competing with respect to our products with companies that have significantly more experience and expertise in undertaking preclinical testing and human clinical trials with new or improved therapeutic products and obtaining regulatory approvals of such products. A number of these companies already market and may be in advanced phases of clinical testing of various drugs that will or may compete with our current product candidates or other future potential product candidates. Our competitors may develop or commercialize products more rapidly than we do or with significant advantages over any products we develop. Our competitors may therefore be more successful in commercializing their products than we are, which could adversely affect our competitive position and business.

In addition to larger pharmaceutical or biopharmaceutical companies that may develop different competing technologies or technologies within the transplant field, we will be competing with a number of smaller biotechnology companies that are focused on transplant technologies, which may include among others Gamida Cell Ltd., Nohla Therapeutics, Inc., and ExCellThera Inc. We are aware of Novartis' collaboration with Intellia Therapeutics, Inc. which includes efforts relating to expansion of HSCs that have been modified using CRISPR/Cas9 technology to express therapeutic proteins and delivered to patients for the treatment of potential treatment of blood disorders or primary immune deficiencies. Any programs and technology that develop as a result of this collaboration would likely compete directly with our E478 program.

Colleges, universities, governmental agencies and other public and private research organizations are becoming more active in seeking patent protection and licensing arrangements to collect royalties for use of technologies that they have developed, some of which may be directly competitive with our programs and product candidates, including our lead product candidate, MGTA-456.

Risks Related to Manufacturing and Commercialization

We rely on third parties to conduct our preclinical and clinical trials and will rely on them to perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

Although we have recruited a team that has experience with clinical trials, as a company we have no experience in conducting clinical trials. Moreover, we do not have the ability to independently conduct preclinical studies and clinical trials, and we have relied upon, and plan to continue to rely upon medical institutions, clinical investigators, contract laboratories and other third parties, or our CROs, to conduct preclinical studies and future clinical trials for our product candidates. We expect to rely heavily on these parties for execution of preclinical and future clinical trials for our product candidates and control only certain aspects of their activities. Nevertheless, we will be responsible for ensuring that each of our preclinical and clinical trials is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards and our reliance on CROs will not relieve us of our regulatory responsibilities. For any violations of laws and regulations during the conduct of our preclinical studies and clinical trials, we could be subject to warning letters or enforcement action that may include civil penalties up to and including criminal prosecution.

We and our CROs will be required to comply with regulations, including cGCPs for conducting, monitoring, recording and reporting the results of preclinical and clinical trials to ensure that the data and results are scientifically credible and accurate and that the trial patients are adequately informed of the potential risks of participating in clinical trials and their rights are protected. These regulations are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for any drugs in clinical development. The FDA enforces cGCP regulations through periodic inspections of clinical trial sponsors, principal investigators and trial sites. If we or our CROs fail to comply with applicable cGCPs, 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 clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, the FDA will determine that any of our future clinical trials will comply with cGCPs. In addition, our clinical trials must be conducted with product candidates produced in accordance with the requirements in the FDA's current Good Manufacturing Practices, or cGMPs, requirements. Our failure or the failure of our CROs to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process and could also subject us to enforcement action.

Although we intend to design our planned clinical trials for our product candidates, for the foreseeable future CROs will conduct all of our planned clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less day-to-day control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs. If CROs 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, regulatory requirements or for other reasons, any preclinical studies or clinical trials with which such CROs are associated with may be extended, delayed or terminated. In such cases, we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates in the subject indication could be harmed, our costs could increase and our ability to generate revenue could be delayed.

The successful development of biopharmaceuticals and cell-based therapies is highly uncertain.

Successful development of biopharmaceuticals and cell-based therapies is highly uncertain and is dependent on numerous factors, many of which are beyond our control. Stem cell transplant and cell-based therapies that appear promising in the early phases of development may fail to reach the market for several reasons including:

- preclinical study results may show the therapies to be less effective than desired or to have harmful or problematic side effects;
- clinical trial results may show the therapies to be less effective than expected (e.g., the trial failed to meet its primary endpoint) or to have unacceptable side effects or toxicities;
- failure to receive the necessary regulatory approvals or a delay in receiving such approvals. Among other things, such delays may be caused by slow enrollment in clinical trials, length of time to achieve study endpoints, additional time requirements for data analysis, or biologics license application, or BLA, preparation, discussions with the FDA, an FDA request for additional preclinical or clinical data, or unexpected safety or manufacturing issues;
- manufacturing costs, formulation issues, pricing or reimbursement issues, or other factors that make the therapy uneconomical; and
- the proprietary rights of others and their competing products and technologies that may prevent the therapy from being commercialized.

Success in preclinical studies and early clinical trials do not ensure that large-scale clinical trials will be successful. The length of time necessary to complete clinical trials and to submit an application for marketing approval for a final decision by a regulatory authority varies significantly from one therapy to the next, and may be difficult to predict.

Even if we are successful in getting market approval, commercial success of any of our product candidates will also depend in large part on the availability of coverage and adequate reimbursement from third-party

payers, including government payers such as the Medicare and Medicaid programs and managed care organizations, which may be affected by existing and future health care reform measures designed to reduce the cost of health care. Third-party payers could require us to conduct additional studies, including post-marketing studies related to the cost effectiveness of a product, to qualify for reimbursement, which could be costly and divert our resources. If government and other health care payers were not to provide adequate coverage and reimbursement levels for any of our products if approved, market acceptance and commercial success would be reduced.

In addition, if one of our product candidates is approved for marketing, we will be subject to significant regulatory obligations regarding the submission of safety and other post-marketing information and reports and registration, and will need to continue to comply (or ensure that our third party providers) comply with the FDA's cGMPs and cGCPs requirements for any clinical trials that we conduct post-approval. In addition, there is always the risk that we or a regulatory authority might identify previously unknown problems with a product post-approval, such as adverse events of unanticipated severity or frequency. Compliance with these requirements is costly, and any failure to comply or other issues with our product candidates' post-market approval could have a material adverse effect on our business, financial condition and results of operations.

We may never obtain FDA approval for any of our product candidates in the United States, and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to eventually market any of our product candidates in any particular foreign jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a jurisdiction-by-jurisdiction basis regarding safety and efficacy. Approval by the FDA in the United States, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. The foreign regulatory approval process involves all of the risks associated with FDA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our products will be unrealized.

MGTA-456 has been affected by contamination issues in the past, and any future contamination in our or our third parties' manufacturing process, shortages of raw materials or reagents or failure of any of our key suppliers to deliver necessary components of our product candidates could result in delays in our clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination. For example, prior to our 2017 license of the product candidate from Novartis, our third-party manufacturer for MGTA-456 experienced contaminations, including microbial contaminations, in connection with the clinical manufacture of MGTA-456 which required disposal of contaminated product and led to delays in the manufacturing process. While we have not experienced contamination events in connection with the manufacture of MGTA-456 for our clinical use since licensing the product candidate, we cannot guarantee that we or our third-party vendors will be able to successfully prevent and remediate contaminations in the future in connection with the manufacture of MGTA-456 or our other current or future product candidates. Any contamination could materially adversely affect our or our third-party vendors' ability to produce our product candidates on schedule and could therefore harm our results of operations and cause reputational damage.

The raw materials required in our and our third-party vendors' manufacturing processes are derived from biological sources. We cannot assure you that we or our third-party vendors have, or will be able to obtain on commercially reasonable terms, or at all, sufficient rights to these materials derived from biological sources. Such raw materials are difficult to procure and may also be subject to contamination or recall or be of insufficient quality. For example, if a selected umbilical cord blood unit is of insufficient quality for manufacture of MGTA-456, we may experience a delay in our clinical trial while MGTA-456 is manufactured using an alternative, back-up umbilical cord blood unit. A material shortage, contamination, recall, or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the clinical and commercial manufacturing of our product candidates, which could materially and adversely affect our operating results and development timelines.

We rely on third-party suppliers for the supply and manufacture of certain components of our technology and product candidates, including a single supplier in some cases. For example, an affiliate of the University of Minnesota is our only manufacturing partner for MGTA-456, and Bachem Americas, Inc. is currently the sole manufacturer of MGTA-145. Should our ability to procure the necessary components for our product candidates from our suppliers be compromised, our ability to continuously operate would be impaired until an alternative supplier is sourced, qualified and tested, which could delay or limit our ability to produce a clinical and commercial supply of our product candidates and harm our business.

If we use biological materials in a manner that causes injury, we may be liable for damages.

Our research and development activities involve the use of biological and hazardous materials. Although we believe our safety procedures for handling and disposing of these materials complies with federal, state and local laws and regulations, we cannot entirely eliminate the risk of accidental injury or contamination from the use, storage, handling or disposal of these materials. We do not carry specific biological waste insurance coverage, workers compensation or property and casualty and general liability insurance policies that include coverage for damages and fines arising from biological exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended or terminated.

Third-party manufacturers and any third-party collaborators may be unable to successfully scale-up manufacturing of MGTA-456 or our other current or future product candidates in sufficient quality and quantity, which would delay or prevent us from developing MGTA-456 or our other product candidates and commercializing approved products, if any.

In order to conduct clinical trials of MGTA-456 and our other current and future product candidates, we will need to work with third-party manufacturers to manufacture them in sufficient quantities. Our manufacturing partners or our third-party collaborators may be unable to successfully increase the manufacturing capacity of MGTA-456 and our other current or future product candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities. If our manufacturing partners or collaborators are unable to successfully scale up the manufacture of our current or future product candidates in sufficient quality and quantity, the development, testing, and clinical trials of that product candidate may be delayed or infeasible, and marketing approval or commercial launch of any resulting product may be delayed or not obtained, which could significantly harm our business.

The commercial success of any of our product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Even with the requisite approvals from the FDA in the United States, the EMA in the European Union and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of our product candidates as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians,

patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy, durability and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the EMA;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and adequate reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

If we are unable to successfully develop our current programs into a comprehensive portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates.

We are developing our product candidates so that they can each be used individually or in combination with each other. In particular, we are focused on a product development strategy that includes leveraging the synergies among a comprehensive portfolio of our product candidates. Our success may depend, in part, on our ability to develop a complementary product portfolio with product candidates that, together or individually, will address the major unmet needs inherent to the existing stem cell transplant process. Given our limited experience in developing product candidates that have received marketing approval, we may not be successful in developing some of our product candidates. The failure of one of our product candidates to obtain regulatory approval or market acceptance may affect our ability to expand our market opportunities for our other product candidates or programs. Although we may develop product candidates that ultimately obtain marketing approval, if we are unable to successfully develop our current programs into a comprehensive portfolio of product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our current and future product candidates.

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

The success of our product candidates, if approved, depends on the availability of adequate coverage and reimbursement from third-party payors. In addition, because our product candidates represent new approaches to

stem cell transplant, we cannot be sure that coverage and reimbursement will be available for, or accurately estimate the potential revenue from our product candidates or assure that coverage and reimbursement will be available for any product that we may develop.

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 U.S., no uniform policy of coverage and reimbursement for products exists among third-party payors. As a result, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical and cost-effectiveness data for the use of our products on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. Even if we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Additionally, third-party payors may not cover, or provide adequate reimbursement for, long-term follow-up evaluations required following the use of product candidates. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates. Because our product candidates may 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. There is significant uncertainty related to insurance coverage and reimbursement of newly approved products. It is difficult to predict at this time what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Middle Class Tax Relief and Job Creation Act of 2012 required that the Centers for Medicare & Medicaid Services, the agency responsible for administering the Medicare program, or CMS, reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which served as a base for 2014 and subsequent years. In addition, effective January 1, 2014, CMS also began bundling the Medicare payments for certain laboratory tests ordered while a patient received services in a hospital outpatient setting. Additional state and federal healthcare reform measures are expected to 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 certain pharmaceutical products or additional pricing pressures.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. There has been increasing legislative and enforcement interest in the United States with respect to

specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, cost containment initiatives and additional legislative changes.

Ongoing healthcare legislative and regulatory reform measures may have a material adverse effect on our business and results of operations.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Affordable Care Act, or the ACA, was passed, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The ACA, among other things, subjects biological products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and creates a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts (increased from 50% effective January 1, 2019) off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Some of the provisions of the ACA have yet to be fully implemented, while certain provisions have been subject to judicial and Congressional challenges, as well as efforts by the Trump administration to repeal or replace certain aspects of the ACA. For example, in Congress, the U.S. House of Representatives passed ACA replacement legislation known as the American Health Care Act of 2017 in May 2017, which was not introduced in the Senate. However, The Tax Cuts and Jobs Act of 2017, or TCJA, includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, ruled that the individual mandate is a critical and inseverable feature of the ACA, and therefore, because it was repealed as part of the TCJA, the remaining provisions of the ACA are invalid as well. The current administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal. Congress may consider other legislation to repeal or replace certain elements of the ACA. In addition, since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Further, the Trump administration has concluded that cost-sharing reduction, or CSR, payments to insurance companies required under the ACA have not received necessary appropriations from Congress and announced that it will discontinue these payments immediately until such appropriations are made. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the ACA. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give

states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. On August 2, 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 of 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2027 unless additional congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers.

These laws, and future state and federal healthcare reform measures may be adopted in the future, any of which 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.

European Union drug marketing and reimbursement regulations may materially affect our ability to market and receive coverage for our products in the European member states.

We intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the European Union, the pricing of biologics is subject to governmental control and other market regulations which could put pressure on the pricing and usage of our product candidates. 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.

Much like the Anti-Kickback Statue prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the European Union. The provision of benefits or advantages to physicians is governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

In addition, in most foreign countries, including the European Economic Area, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing and reimbursement vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced

member states, can further reduce prices. A member state may approve a specific price for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, we may be required to conduct a clinical study or other studies that compare the cost-effectiveness of any of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States and generally prices tend to be significantly lower. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If pricing is set at unsatisfactory levels or if reimbursement of our products is unavailable or limited in scope or amount, our revenues from sales by us or our strategic partners and the potential profitability of any of our product candidates in those countries would be negatively affected.

European data collection is governed by restrictive regulations governing the use, processing, and cross-border transfer of personal information.

The collection and use of personal health data in the European Union is governed by the provisions of the Data Protection Directive, and as of May 2018 the General Data Protection Regulation, or GDPR. This directive imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive and GDPR also impose strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with the requirements of the Data Protection Directive, the GDPR, and the related national data protection laws of the European Union Member States may result in fines and other administrative penalties. The GDPR introduces new data protection requirements in the European Union and substantial fines for breaches of the data protection rules. Notably, on January 21, 2019, Google was fined almost $57 million by French regulators for violating GDPR. The GDPR regulations may impose additional responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and adversely affect our business, financial condition, results of operations and prospects.

Laws and regulations governing international operations may preclude us from developing, manufacturing and selling certain products outside of the United States and require us to develop, implement and maintain costly compliance programs.

If we further expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or 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.

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

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products 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 civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We currently have no marketing and sales organization and have no experience in marketing products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate product revenue.

We currently have no sales, marketing or distribution capabilities and have no experience in marketing products. We intend to develop an in-house marketing organization and sales force, which will require significant capital expenditures, management resources and time. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel.

If we are unable or decide not to establish internal sales, marketing and distribution capabilities, we will pursue collaborative arrangements regarding the sales and marketing of our products, however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements, or if we are able to do so, that they will have effective sales forces. Any revenue we receive will depend upon the efforts of such third parties, which may not be successful. We may have little or no control over the marketing and sales efforts of such third parties and our revenue from product sales may be lower than if we had commercialized our product candidates ourselves. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates.

There can be no assurance that we will be able to develop in-house sales and distribution capabilities or establish or maintain relationships with third-party collaborators to commercialize any product in the United States or overseas.

Even if we are able to commercialize any product candidates, such products may become subject to unfavorable pricing regulations, third-party reimbursement practices, or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing, and reimbursement for new medicines vary widely from country to country. In the United States, recently enacted legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a medicine before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a medicine in a particular country, but then be subject to price regulations that delay our commercial launch of the medicine, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the medicine in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if any product candidates we may develop obtain marketing approval.

Our ability to commercialize any medicines successfully also will depend in part on the extent to which reimbursement for these medicines and related treatments will be available from government health administration authorities, private health insurers, and other organizations. Government authorities and third-

party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. For example, the Trump administration released a "Blueprint," or plan, to reduce the cost of drugs. The Trump administration's Blueprint contains certain measures that the U.S. Department of Health and Human Services is already working to implement. For example, on October 25, 2018, CMS issued an Advanced Notice of Proposed Rulemaking, or ANPRM, indicating it is considering issuing a proposed rule in the spring of 2019 on a model called the International Pricing Index, or IPI. This model would utilize a basket of other countries' prices as a reference for the Medicare program to use in reimbursing for drugs covered under Part B. The ANPRM also included an updated version of the Competitive Acquisition Program as an alternative to current "buy and bill" payment methods for Part B drugs. Individual state legislatures have become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing. Some of these measures include price or patient reimbursement constraints, discounts, restrictions on certain product access, marketing cost disclosure and transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. Also, increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any medicine that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved medicines, and coverage may be more limited than the purposes for which the medicine is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any medicine will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution. Interim reimbursement levels for new medicines, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the medicine and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost medicines and may be incorporated into existing payments for other services. Net prices for medicines may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of medicines from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved medicines we may develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize medicines, and our overall financial condition.

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

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

to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

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

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop, alone or with collaborators. Although we currently carry $10 million of clinical trial insurance, the amount of such insurance coverage may not be adequate, we may be unable to maintain such insurance, or we may not be able to obtain additional or replacement insurance at a reasonable cost, if at all. Our insurance policies may also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our third-party collaborators 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 regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Foreign governments often impose strict price controls on approved products, which may adversely affect our future profitability in those countries, and the re-importation of drugs to the United States from foreign countries that impose price controls may adversely affect our future profitability.

Frequently foreign governments impose strict price controls on newly approved therapeutic products. If we obtain regulatory approval to sell products in foreign countries, we may be unable to obtain a price that provides an adequate financial return on our investment. Furthermore, legislation in the United States may permit re-importation of drugs from foreign countries into the United States, including re-importation from foreign countries where the drugs are sold at lower prices than in the United States due to foreign government-mandated price controls. Such a practice, especially if it is conducted on a widespread basis, may significantly reduce our potential United States revenues from any drugs that we are able to develop.

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

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising, and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

Accordingly, assuming we, or any collaborators we may have, receive marketing approval for one or more product candidates we develop, we, and such 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 such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products 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 business, operating results, financial condition, and prospects.

Risks Related to Intellectual Property

We are highly dependent on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.

In April 2017, we entered into a license agreement with Novartis pursuant to which we were granted a worldwide license to certain intellectual property rights owned or controlled by Novartis, including patents, patent applications, proprietary information, know-how and other intellectual property, to develop, commercialize and sell one or more therapeutic products comprising MGTA-456 in the field of non-gene-edited/-modified HSCs. In addition, in November 2016, we entered into a license agreement with Harvard University, or Harvard, pursuant to which we were granted a worldwide license to research, develop and commercialize one or more therapeutic products under certain conditioning- and mobilization-related patents and patent applications owned or controlled by Harvard. Furthermore, in March 2018, we entered into a research, development option and license agreement with Heidelberg Pharma Research GmbH, or Heidelberg Pharma, pursuant to which we intend to combine our proprietary antibodies and Heidelberg Pharma's amanitin conjugates platform. We are

dependent on the patents, know-how and proprietary technology, licensed from Novartis and Harvard. Furthermore, if we commercialize any products utilizing Heidelberg Pharma's amanitin conjugates platform, we will be dependent on the intellectual property rights we license from Heidelberg Pharma. Any termination of these licenses, or a finding that such intellectual property lacks legal effect, could result in the loss of significant rights and could harm our ability to commercialize MGTA-456, C100, C200, C300, G100, MGTA-145, and other product candidates.

Certain of our license agreements, including our agreements with Novartis, Harvard and Heidelberg Pharma, require us to use diligent efforts or meet development thresholds, to maintain the license, including establishing a set timeline for developing and commercializing products. If we fail to comply with the obligations under our license agreements, including payment terms and diligence terms, our licensors may have the right to terminate our agreements, in which event we may not be able to develop, manufacture, market or sell the products covered by our agreements or may face other penalties under our agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of our license agreements or reduction or elimination of our rights under them may result in our having to negotiate a new or reinstated agreement, which may not be available to us on equally favorable terms, or at all, which may mean we are unable to develop or commercialize the affected product candidate or cause us to lose our rights under the agreement. In addition, with respect to our license agreement with Novartis, Novartis has granted an exclusive license to Intellia Therapeutics, Inc., or Intellia, in the field of gene-modified HSCs under the same intellectual property that Novartis licensed to us. Accordingly, such rights are unavailable to us and in prosecuting, maintaining, enforcing and defending the licensed patents, Novartis may make decisions that may not be in our best interest. Moreover, if Novartis or Intellia take any action with respect to the licensed patents that results in a successful challenge to the licensed patents by any third party, such patents may be invalidated or held to be unenforceable and we may lose our rights under such patents, which would harm our business.

Further, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. Accordingly, disputes may arise between us and our licensor, our licensor and its licensors, regarding intellectual property subject to a license agreement, including those relating to:

- the scope of rights, if any, granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the license agreement;
- whether our licensor or its licensor had the right to grant the license agreement;
- whether third parties are entitled to compensation or equitable relief, such as an injunction, for our use of the intellectual property without their authorization;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- whether we are complying with our obligations with respect to the use of the licensed technology in relation to our development and commercialization of product candidates;
- our involvement in the prosecution of the licensed patents and our licensors' overall patent enforcement strategy;
- the allocation of ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and by us and our partners; and
- the amounts of royalties, milestones or other payments due under the license agreement.

The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be

our financial or other obligations under the relevant agreement. If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, or are insufficient to provide us the necessary rights to use the intellectual property, we may be unable to successfully develop and commercialize the affected product candidates. If we or any such licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer. Any disputes with our licensors or any termination of the licenses on which we depend could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our commercial success depends on our ability to obtain, maintain and protect our intellectual property and proprietary technology.

Our commercial success depends in large part on our ability to obtain, maintain and protect intellectual property protection through patents, trademarks, and trade secrets in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode, negate or preempt any competitive advantage we may have, which could harm our business and ability to achieve profitability.

To protect our proprietary position, we have submitted patent applications and may file other patent applications in the United States or abroad related to our product candidates that are important to our business. Although we in-license certain issued patents from Novartis related to our MGTA-456 product candidate, we own only two issued U.S. patents related to our product candidates and many of the patent applications that we own in the United States are provisional patent applications. Provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of the filing of one or more of our related provisional patent applications. If we do not timely file any non-provisional patent applications, we may lose our priority date with respect to our provisional patent applications and any patent protection on the inventions disclosed in our provisional patent applications. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether any such patent applications will result in the issuance of patents that provide us with any competitive advantage. Moreover, the patent application and approval process is expensive and time-consuming. 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 some instances, agreements through which we license patent rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented, how claims are amended, and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, including under our agreement with Novartis, and therefore cannot guarantee that these patents and applications will be prosecuted or maintained in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents. Moreover, some of our in-licensed patents and patent applications are, and our future owned and licensed patents may be, co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us.

If the scope of the patent protection we or our licensors obtain is not sufficiently broad, we may not be able to prevent others from developing and commercializing technology and products similar or identical to ours. The degree of patent protection we require to successfully compete in the marketplace may be unavailable or severely limited in some cases and may not adequately protect our rights or permit us to gain or keep any competitive

advantage. We cannot provide any assurances that any of our licensed patents have, or that any of our pending owned or licensed patent applications that mature into issued patents will include, claims with a scope sufficient to protect our product candidates or otherwise provide any competitive advantage, nor can we assure you that our licenses will remain in force. Other parties have developed or may develop technologies that may be related or competitive with our approach, and may have filed or may file patent applications and may have been issued or may be issued patents with claims that overlap or conflict with our patent applications, either by claiming the same compounds, formulations or methods or by claiming subject matter that could dominate our patent position. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Various extensions may be available; however, the life of a patent, and the protection it affords, is limited. 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 patent portfolio may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar to our product candidates. In addition, the patent portfolio licensed to us is, or may be, licensed to third parties, such as outside our field, and such third parties may have certain enforcement rights. Thus, our owned and licensed patents and any patents we own or license in the future could be put at risk of being invalidated or interpreted narrowly in litigation filed by or against another licensee or in administrative proceedings brought by or against another licensee in response to such litigation or for other reasons.

The patent protection we obtain for our product candidates may not be sufficient enough to provide us with any competitive advantage or our patents may be challenged.

Our owned and licensed patents and pending patent applications, if issued, may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patents by developing similar or alternative technologies or therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapy that provides benefits similar to one or more of our product candidates but falls outside the scope of our patent protection or license rights. If the patent protection provided by the patents and patent applications we hold or pursue with respect to our product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product candidates could be negatively affected, which would harm our business. Currently, a significant portion of our patents and patent applications are in-licensed, though similar risks would apply to any patents or patent applications that we now own or may own or in-license in the future.

We, or any future partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or requests for patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors, are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

The patent position of biotechnology and pharmaceutical companies carries uncertainty. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which are dependent upon the current legal and intellectual property context, extant legal

precedent and interpretations of the law by individuals. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are characterized by uncertainty.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are 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. Similarly, we cannot be certain that parties from whom we do or may license or purchase patent rights were the first to make relevant claimed inventions, or were the first to file for patent protection for them. If third parties have filed prior patent applications on inventions claimed in our patents or applications that were filed on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such prior applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our owned and licensed patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivation proceedings, *ex parte* reexaminations, *inter partes* review, supplemental examinations, or interference proceedings or challenges in district court, in the United States or in various foreign patent offices, including both national and regional, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of the patent or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, or in denial of the patent application or loss or reduction in the scope of one or more claims of the patent application, any of which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, 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.

Pending and future patent applications may not result in patents being issued that protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Competitors may also be able to design around our patents. 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 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 or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than United States law does. If these developments were to occur, they could have a material adverse effect on our ability to generate revenue.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our future development partners will be successful in protecting our product candidates by obtaining and defending patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can 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;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable or otherwise may not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with or eliminate our ability to make, use, and sell our potential product candidates;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates.

Issued patents that we have or may obtain or license may not 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 patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting Abbreviated New Drug Applications, or ANDAs, to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position may be harmed.

In addition to the protection afforded by patents, we rely upon trade secret protection, know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, scientific advisors, employees and consultants and invention assignment agreements with our consultants and employees. However, we may not obtain these agreements in all circumstances, and individuals with whom we have these agreements may not comply with their terms. The assignment of intellectual property rights under these agreements may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. In addition, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements despite the existence of confidentiality agreements and other contractual restrictions. Monitoring

unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, scientific advisors, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim against a third party that illegally obtained and is using our trade secrets, like patent litigation, is expensive and time-consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing or unwilling to protect trade secrets.

Moreover, our trade secrets could otherwise become known or be independently discovered by our competitors or other third parties. Competitors and other third parties could purchase our product candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

Third-party claims of intellectual property infringement, misappropriation or other violations may prevent or delay our product discovery and development efforts and have a material adverse effect on our business.

Our commercial success depends in part on our avoiding infringement, misappropriation and other violations of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including *inter partes* review and post grant review have been implemented. As stated above, this reform will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others.

Third parties may assert that we are employing their proprietary technology without authorization. For example, we are aware of certain patent applications owned by a third party with claims that if issued in their present form could be construed to cover C200. If such patent claims are issued, the third party may seek to allege that our development and commercialization of C200 infringes such patents and file a patent infringement lawsuit against us in the future. While we believe we would have valid defenses against any such allegation or lawsuit, such defenses may be unsuccessful. In this regard, patents issued in the U.S. by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof. There may also be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, or any final product itself, the holders of any such patents may be able to block our ability to commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. Similarly, if any third-party patent were held by a court of

competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy or patient selection methods, the holders of any such patent may be able to block our ability to develop and commercialize the product candidate unless we obtained a license or until such patent expires or is finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. Even if we obtained such a license, it may only be non-exclusive, which would permit third parties to use the same intellectual property and compete with us. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, we may be unable to commercialize our product candidates or such efforts may be impaired or delayed, which could in turn significantly harm our business.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. We may not have sufficient resources to bring these actions to a successful conclusion. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock.

In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could harm our business significantly. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

Obtaining and maintaining our patent protection depends on 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.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market earlier than would otherwise have been the case, which would have a material adverse effect on our business.

Some intellectual property that we have in-licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S.-based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Many of the intellectual property rights we have licensed are generated through the use of U.S. government funding and are therefore subject to certain federal regulations. As a result, the U.S. government may have

certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive

as a result of the proceedings. Any of the foregoing may have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents.

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

Filing, prosecuting, maintaining, defending and enforcing patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and may export otherwise infringing drugs to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These drugs may compete with our product candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful.

Many countries have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license, which could adversely affect our business, financial condition, results of operations, and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the U.S., if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from competitive medications, including generic medications. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours.

Depending upon the timing, duration and conditions of any FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. However, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. Only one patent per approved product can be extended, the extension cannot extend the total patent term beyond 14 years from approval and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for the applicable product candidate will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case, and our competitive position, business, financial condition, results of operations, and prospects could be materially harmed.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

We employ individuals who were previously employed at universities or other biopharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. 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 could result in substantial costs and be a distraction to management and other employees.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make products that are similar to any product candidates we may develop or utilize similar technology but that are not covered by the claims of the patents that we license or own;

- we, or our current or future licensors might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or own;
- we, or our current or future licensors might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending owned or licensed patent applications or those that we may own or license in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent in order to maintain certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could harm our business, financial condition, results of operations, and prospects.

Risks Related to Our Dependence on Third Parties

We expect to depend on collaborations with third parties for the research, development, and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We anticipate seeking third-party collaborators for the research, development, and commercialization of certain of the product candidates we may develop. Our likely collaborators for any other collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies, and biotechnology companies. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop, pose the following risks to us:

- Collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations.
- Collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development 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, products that compete directly or indirectly with our medicines or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours.
- Collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines.
- Collaborators may not properly obtain, maintain, enforce, or defend our intellectual property or proprietary 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.
- Disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or product candidates or that result in costly litigation or arbitration that diverts management attention and resources.
- We may lose certain valuable rights under circumstances identified in our collaborations, including if we undergo a change of control.
- Collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.
- Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished, or terminated.

If our collaborations do not result in the successful development and commercialization of products, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval, and commercialization described in this Annual Report on Form 10-K apply to the activities of our collaborators.

We have in the past and may in the future decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of any product candidates we may develop. These relationships, or those like them, may require us to incur non-recurring and 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 could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement 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 several factors. If we license rights to any product candidates we or our collaborators may develop, 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.

We are developing E478 specifically to partner with gene therapy and genome editing companies. If we are unable to find willing collaborators, this may adversely affect the development of E478 and our business.

We are developing E478 specifically to partner and collaborate with gene therapy and genome editing companies. In particular, we seek to selectively pursue collaboration arrangements with companies that have particular technology, expertise or resources for the development of E478, if approved. However, we may not be

able to execute on such collaboration and any collaboration that we may enter into may not be successful. If we are unable to identify partners whose capabilities complement and integrate well with ours and reach collaboration arrangements with such partners on a timely basis, on acceptable terms or at all, or if the arrangements we establish are unproductive for us, we may fail to meet our business and development objectives for E478, which may adversely affect our business.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates. For example, in connection with our license agreement with Novartis, we issued to Novartis, 2,500,000 shares of Series A redeemable convertible preferred stock and 643,550 shares of Series B redeemable convertible preferred stock, causing our stockholders to experience dilution. If in the future, we enter into collaborations with other third parties, we may issue additional equity as part of such collaboration.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

If any party to which we have outsourced certain functions fails to perform its obligations under agreements with us, the development and commercialization of our product candidates and any future product candidates could be delayed or terminated.

To the extent that we rely on third party individuals or other companies to manage the day-to-day conduct of our clinical trials or to manufacture, sell or market our current product candidates or any future product candidates, we will be dependent on the timeliness and effectiveness of their efforts. If a clinical research management organization that we might utilize is unable to allocate sufficient qualified personnel to our studies or if the work performed by it does not fully satisfy the rigorous requirements of the FDA, we may encounter substantial delays and increased costs in completing our clinical trials. If a firm producing humanized forms of our molecular antibody product candidates or a manufacturer of the raw material or finished product for our clinical trials is unable to meet our time schedules or cost parameters, the timing of our clinical trials and development of our product candidates may be adversely affected. Any manufacturer that we select may encounter difficulties in scaling-up the manufacture of new products in commercial quantities, including problems involving product yields, product stability or shelf life, quality control, adequacy of control procedures and policies, compliance with FDA regulations and the need for further FDA approval of any new manufacturing processes and facilities. The manufacture of clinical supplies for studies and commercial quantities of our current product candidates and any future product candidates are likely to be inherently more difficult and costly than

typical chemical pharmaceuticals. This could delay commercialization of any of our product candidates, if approved, or reduce the profitability of these candidates for us. If any of these occur, the development and commercialization of our product candidates could be delayed, curtailed or terminated because we may not have sufficient financial resources or capabilities to continue such development and commercialization on our own.

We expect to continue to rely on third parties to manufacture our clinical product supplies, and we intend to rely on third parties to produce and process our product candidates, if approved.

We do not currently own any facility that may be used as our clinical-scale manufacturing and processing facility and must eventually rely on outside vendors to manufacture supplies and process our product candidates. We have not yet caused any product candidates to be manufactured or processed on a commercial scale and may not be able to do so for any of our product candidates. We will make changes as we work to optimize the manufacturing process, and we cannot be sure that even minor changes in the process will result in therapies that are safe and effective.

The facilities used by our contract manufacturers to manufacture our product candidates must be approved by the FDA or other foreign regulatory agencies pursuant to inspections that will be conducted after we submit an application to the FDA or other foreign regulatory agencies. We do not control the manufacturing process of, and will be completely dependent on, our contract manufacturing partners for compliance with regulatory requirements, known as cGMP requirements for manufacture of our product candidates. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our product candidates, if approved.

In addition, should any of our agreements with our contract manufacturers terminate, in particular the agreements with the University of Minnesota and Heidelberg Pharma, they may be difficult to replace if we were no longer able to rely on them.

Risks Related to Employee Matters, Managing Growth and Other Risks Related to Our Business

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of December 31, 2018, we had 59 full time employees. As our development, manufacturing and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need and are actively recruiting additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining and motivating additional employees;
- managing our internal development efforts effectively, including the clinical, FDA and international regulatory review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to develop, manufacture and commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert financial and other resources, and a disproportionate amount of their attention away from day-to-day activities in order to devote a substantial amount of time, to managing these growth activities.

We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including substantially all aspects of regulatory approval, clinical management and manufacturing. We cannot assure you that the services of independent organizations, advisors and consultants will continue to be available to us on a timely basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. We cannot assure you that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

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

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational, and financial systems, expand our facilities, and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expected expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected 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.

If we lose key management personnel, or if we fail to recruit additional highly skilled personnel, our ability to identify and develop new or next generation product candidates will be impaired, which could result in loss of market opportunities or market share and could make us less competitive.

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, particularly our Chief Executive Officer, the members of our executive team, and key scientific and medical personnel employees. The loss of the services of any of our executive officers, key employees, and scientific and medical advisors, and our inability to find suitable replacements, could result in delays in product development and harm our business.

We conduct our operations at our facility in Cambridge, Massachusetts. This region is headquarters to many other biopharmaceutical companies and many academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with our key employees, these agreements provide for at-will employment, which means that any of our employees could leave our employment at any

time, with or without notice. We do not maintain "key man" insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. 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 conducted in connection with Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls 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 stock.

We will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an "emerging growth company" under the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act. We could be an "emerging growth company" for up to five years. An independent assessment of the effectiveness of our internal controls over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal controls over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As has been widely reported, global credit and financial markets have experienced extreme volatility and disruptions in the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by any such economic downturn, volatile business environment or continued unpredictable and unstable market conditions. If the current equity and credit markets deteriorate, or do not improve, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget.

At December 31, 2018, we had $142.6 million of cash, cash equivalents and marketable securities. While we are not aware of any downgrades, material losses, or other significant deterioration in the fair value of our cash equivalents or marketable securities since December 31, 2018, no assurance can be given that further deterioration of the global credit and financial markets would not negatively impact our current portfolio of cash

equivalents or our ability to meet our financing objectives. Furthermore, our stock price may decline due in part to the volatility of the stock market and the general economic downturn.

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

Our operations, and those of our CMOs, our CROs and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. 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.

Our internal computer systems, or those used by our CMOs, CROs or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our future CMOs, future CROs and other contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. If such a system failure or security breach 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 may rely on 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, including potential lawsuits from patients, collaborators, employees and/or stockholders, and the further development and commercialization of our product candidates could be delayed.

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

Social media is increasingly being used to communicate about our clinical development programs and the diseases our therapeutics are being developed to treat, and we intend to utilize appropriate social media in connection with our commercialization efforts following approval of our product candidates, if any. 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. 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. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event 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 product candidates. 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.

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 employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional,

reckless and/or negligent conduct that fails to comply with the laws of the FDA and other similar foreign regulatory bodies, provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies, comply with manufacturing standards we have established, comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws, or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs.

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

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of pharmaceutical products. Arrangements with third-party payors and customers can expose pharmaceutical manufacturers to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which such companies sell, market and distribute pharmaceutical products. 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. The applicable federal, state and foreign healthcare laws and regulations laws that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity can be found guilty of violating the statute without actual knowledge of the statute or specific intent to violate it. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the False Claims Act, or FCA, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, false or fraudulent claims for payment to, or approval by Medicare, Medicaid, or other federal healthcare programs, knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim or an obligation to pay or transmit money to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government. Manufacturers can be held liable under the FCA even when they do not submit claims directly to government payors if they are deemed to "cause" the submission of false or fraudulent claims. The FCA also permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the FCA and to share in any monetary recovery;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose, among other things, requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;
- the federal Physician Payment Sunshine Act, created under the Health Reform Law, and its implementing regulations, which require manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the United States Department of Health and Human Services, or HHS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Additionally, we are subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope and may apply regardless of the payor.

Pricing and rebate programs must comply with the Medicaid rebate requirements of the U.S. Omnibus Budget Reconciliation Act of 1990 and more recent requirements in the ACA. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. Manufacturing, sales, promotion and other activities also are potentially subject to federal and state consumer protection and unfair competition laws.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies often scrutinize interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry. Ensuring business arrangements comply with applicable healthcare laws, as well as

responding to possible investigations by government authorities, can be time- and resource-consuming and can divert a company's attention from the business.

The failure to comply with any of these laws or regulatory requirements subjects entities to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, possible exclusion from participation in federal and state funded healthcare programs, contractual damages and the curtailment or restricting of our operations, as well as additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws. Any action for violation of these laws, even if successfully defended, could cause a pharmaceutical manufacturer to incur significant legal expenses and divert management's attention from the operation of the business. Prohibitions or restrictions on sales or withdrawal of future marketed products could materially affect business in an adverse way. In connection with our IPO, we adopted a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct 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 be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and 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 civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

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

As of December 31, 2018, we had net operating loss carryforwards for federal income tax purposes of $40.6 million, of which $17.5 million begin to expire in 2035 and $23.1 million can be carried forward indefinitely. As of December 31, 2018, we had net operating loss carryforwards for state income tax purposes of $40.8 million which begin to expire in 2035. As of December 31, 2018, we also had available research and orphan drug tax credit carryforwards for federal and state income tax purposes of $3.4 million and $0.8 million, respectively, which begin to expire in 2035 and 2030, respectively. Utilization of the net operating loss carryforwards and research and orphan drug tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended (the "Code") due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. The Company has not conducted a formal study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If an ownership change has occurred or does occur in the future, the amount of net operating loss and tax credit carryforwards presented in our financial statements could be limited or expire unutilized.

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing or competing in the market for certain indications.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our

resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. 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 collaboration, 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. and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us.

We may also be restricted under existing collaboration agreements from entering into future agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, 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 product revenue.

In addition, any collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of the collaboration arrangement. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

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 is characterized by intense competition and rapid innovation. Our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and 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 and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that

are more effective, safer, more easily commercialized or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement.

Our competitors include companies focused on developing technologies to improve the distinct steps of stem cell transplant.

Competitors in our stem cell expansion programs include: Gamida Cell Ltd., Nohla Therapeutics, Inc., ExCellThera Inc., Angiocrine Bioscience, Inc. and Intellia Therapeutics, Inc. In particular, Intellia Therapeutics, Inc. has exclusively licensed from Novartis the aryl hydrocarbon receptor antagonist that we use to manufacture MGTA-456 for expansion of gene-modified HSCs only, and it is likely that the programs developed under this license would compete directly with our E478 program.

We also face competition in our conditioning programs from Actinium Pharmaceuticals, Inc., Stanford University, Forty Seven, Inc. and Molecular Templates, Inc., and in our post-transplant complications program (GvHD) from Bellicum Pharmaceuticals, Inc., Kiadis Pharma NV and Abbvie Inc. Additionally, BioLineRx Ltd. is a competitor in our mobilization program.

In addition, we anticipate competing with the largest pharmaceutical companies in the world, such as Novartis, which is currently conducting research relating to expansion of HSCs that have been modified using CRISPR/Cas9 technology to express therapeutic proteins and delivered to patients for the treatment of potential treatment of blood disorders or primary immune deficiencies, which has greater financial and human resources than we currently have.

Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances.

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 the success of 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 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, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. 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 production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. We do not currently carry biological or hazardous waste insurance coverage.

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 or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities.

We may not be successful in our efforts to identify additional product candidates. Due to our limited resources and access to capital, we must prioritize development of certain product candidates; these decisions may prove to be wrong and may adversely affect our business.

Although we intend to explore other therapeutic opportunities, in addition to the product candidates that we are currently developing, we may fail to identify successful product candidates for clinical development for a number of reasons. If we fail to identify additional potential product candidates, our business could be materially harmed.

Research programs to pursue the development of our planned product candidates for additional indications and to identify new product candidates and disease targets require substantial technical, financial and human resources whether or not they are ultimately successful. Our research programs may initially show promise in identifying potential indications and/or product candidates, yet fail to yield results for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential indications and/or product candidates;
- potential product candidates may, after further study, be shown to have harmful adverse effects or other characteristics that indicate they are unlikely to be effective drugs; or
- it may take greater human and financial resources than we will possess to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, thereby limiting our ability to develop, diversify and expand our product portfolio.

Because we have limited financial and human resources, we intend to initially focus on research programs and product candidates for a limited set of indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential or a greater likelihood of success. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Accordingly, there can be no assurance that we will ever be able to identify additional therapeutic opportunities for our product candidates or to develop suitable potential product candidates through internal research programs, which could materially adversely affect our future growth and prospects. We may focus our efforts and resources on potential product candidates or other potential programs that ultimately prove to be unsuccessful.

Risks Related to Our Common Stock

An active trading market for our common stock may not be sustained.

In June 2018, we closed our IPO. Prior to our IPO, there was no public market for our common stock. Although we have completed our IPO and shares of our common stock are listed and trading on The Nasdaq Global Market, an active trading market for our shares may not be sustained. If an active market for our common

stock does not continue, it may be difficult for our stockholders to sell their shares without depressing the market price for the shares or sell their shares at or above the prices at which they acquired their shares or sell their shares at the time they would like to sell. Any inactive trading market for our common stock may also impair our ability to raise capital to continue to fund our operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

The trading price of our common stock is likely to be highly volatile. Securities class action or other litigation involving our company or members of our management team could also substantially harm our business, financial condition and results of operations.

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

- the success of existing or new competitive products or technologies;
- regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- the timing and results of preclinical studies for any of our product candidates;
- the timing and results of clinical trials of MGTA-456 and any other product candidates;
- commencement or termination of collaborations for E478 or any of our current and future programs and product candidates;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results or development timelines;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- disruptions to political, governmental or regulatory systems, including shutdowns of the government and its agencies;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

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 biopharmaceutical companies, which have experienced significant stock price volatility in recent years.

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

We are an emerging growth company, as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we completed our IPO, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the closing of our IPO, (b) in which we have total annual gross revenue of at least $1.07 billion or (c) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed $700 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (1) irrevocably elect to "opt out" of such extended transition period or (2) no longer qualify as an emerging growth company.

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

As a public company, and particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting and other expenses that we did not incur as a private company, including costs associated with public company reporting requirements. We also anticipate that we will incur costs associated with relatively recently adopted corporate governance requirements, including requirements of the SEC, and The Nasdaq Global Market. We expect these rules and regulations to increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We also expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as executive officers.

We are currently evaluating and monitoring developments with respect to these rules, and we cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC after we become a public company. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of any future debt or credit agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Our executive officers, directors and principal stockholders, together with their respective affiliates, beneficially owned approximately 70% of our capital stock as of February 28, 2019. This concentration of ownership control could delay, defer or prevent a change in control, entrench our management or the board of directors, or impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

We have broad discretion over the use of our cash and investments and may not use them effectively.

Our management has broad discretion to use our cash and investments 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 product candidates. Pending our use to fund operations, we may invest our cash and investments in a manner that does not produce income or that loses value.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management or hinder efforts to acquire a controlling interest in us.

Provisions in our corporate charter and our 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 our

stockholders might otherwise receive a premium for their 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 all members of the board are not 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 nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- 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 a special meeting of stockholders;
- 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 66.67% 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, 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. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders. This could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in the best interest of our stockholders. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our amended and restated bylaws provide that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for most legal actions between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Pursuant to our amended and restated bylaws, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of or based on a breach of a fiduciary duty owed by any of our current or former directors, officers or other employees to us or our stockholders, (3) any action asserting a claim against us or any of our current or former directors, officers, employees or stockholders arising pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws or (4) any action asserting a claim governed by the internal affairs doctrine. Our amended and restated bylaws further provide that the United States District Court for the District of Massachusetts will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act of 1933, as amended, or the Securities Act. In addition, our amended and restated bylaws provide that any person or entity purchasing or otherwise acquiring any interest in shares of our common stock is deemed to have notice of and consented to the foregoing provisions. We have chosen the United States District Court for the District of Massachusetts as the exclusive forum for such causes

of action because our principal executive offices are located in Cambridge, Massachusetts. We recognize that the forum selection clauses in our amended and restated bylaws may impose additional litigation costs on stockholders in pursuing any such claims, particularly if the stockholders do not reside in or near the State of Delaware or the Commonwealth of Massachusetts, as applicable. Additionally, the forum selection clauses in our amended and restated bylaws may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders who assert the provision is not enforceable. The Court of Chancery of the State of Delaware or the United States District Court for the District of Massachusetts, as applicable, may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments may be more or less favorable to us than our stockholders.

Some companies that have adopted similar federal district court forum selection provisions are currently subject to a suit in the Court of Chancery of the State of Delaware brought by stockholders who assert that the federal district court forum selection provision is not enforceable. On December 19, 2018, Court of Chancery of the State of Delaware issued a decision in that lawsuit declaring that such federal forum selection provisions purporting to require claims under the Securities Act be brought in federal court are "ineffective and invalid" because such claims are "external to the corporation." On January 17, 2019, the defendants in that lawsuit appealed the Court of Chancery's decision to the Delaware Supreme Court. While the Delaware Supreme Court recently dismissed the appeal on jurisdictional grounds, we expect that the appeal will be re-filed after the Court of Chancery issues a final judgment. Unless and until the Court of Chancery's decision is reversed or otherwise abrogated, the Company will not seek to enforce its federal forum selection provision designating the District of Massachusetts as the exclusive forum for Securities Act claims. As a result of the Court of Chancery's decision or a decision by the Supreme Court of Delaware affirming the Court of Chancery's decision, we may incur additional costs associated with our federal forum selection bylaw provision, which could have an adverse effect on our business, financial condition or results of operations.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common stock may be influenced, in part, by the research and reports that industry or securities analysts publish about us or our business. We do not currently have and may never obtain research coverage by securities and industry analysts. If no or few securities or industry analysts commence coverage of us, or one or more of the analysts who cover us issues an adverse opinion about our company, our stock price would likely decline. If one or more of these analysts ceases research coverage of us or fails to regularly publish reports on us, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

Our headquarters are located at 100 Technology Square, Cambridge, Massachusetts, where we occupy approximately 69,000 square feet of research and development, laboratory and office space. This lease expires in February 2028. We have subleased approximately 27,000 square feet of office space at our headquarters to third parties. The subleases expire in the fourth quarter of 2020. We believe that our office and laboratory space is sufficient to meet our needs for the foreseeable future and that suitable additional space will be available as and when needed.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may be subject to legal proceedings and claims in the ordinary course of business. We are not currently aware of any such proceedings or claims that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

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

Certain Information Regarding the Trading of Our Common Stock

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

Holders of Our Common Stock

As of February 28, 2019, there were approximately 54 holders of record of shares of our common stock. This number does not include stockholders for whom shares are held in "nominee" or "street" name.

Securities Authorized for Issuance Under Equity Compensation Plans

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report.

Recent Sales of Unregistered Securities

None.

Use of Proceeds from Initial Public Offering

On June 25, 2018, we completed the initial public offering of our common stock pursuant to which we issued and sold 6,666,667 shares of our common stock at a price to the public of $15.00 per share.

All of the shares issued and sold in the initial public offering were registered under the Securities Act pursuant to a Registration Statement on Form S-1 (File No. 333-225178), which was declared effective by the SEC on June 20, 2018. Following the sale of the shares in connection with the closing of our initial public offering, the offering terminated. Goldman Sachs & Co. LLC, J.P. Morgan Securities LLC and Cowen & Co. acted as joint book-running managers and Wedbush PacGrow acted as lead manager of our initial public offering.

We received aggregate gross proceeds from our initial public offering of approximately $100.0 million, or aggregate net proceeds of approximately $89.9 million after deducting underwriting discounts and commissions and offering expenses. None of the underwriting discounts and commissions or offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any of our affiliates.

As of December 31, 2018, we estimate that we have used approximately $28 million of the net proceeds from our initial public offering for clinical development of our product candidates and research activities and for working capital and other general corporate purposes. We have not used any of the net proceeds from the offering to make payments, directly or indirectly, to any director or officer of ours, or any of their associates, to any person owning 10 percent or more of our common stock or to any affiliate of ours. We have invested the remaining net proceeds from the offering in money market accounts. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on June 21, 2018.

Issuer Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

ITEM 6. SELECTED FINANCIAL DATA

You should read the following selected financial data together with our consolidated financial statements and the related notes appearing at the end of this Annual Report on Form 10-K and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section. We have derived the statement of operations data for the years ended December 31, 2018, 2017 and 2016 and the balance sheet data as of December 31, 2018 and 2017 from our audited consolidated financial statements appearing at the end of this Annual Report on Form 10-K. The balance sheet data as of December 31, 2016 is derived from our audited consolidated financial statements not included in this Annual Report on Form 10-K. Our historical results are not necessarily indicative of the results that may be expected in any future period.

	Year Ended December 31,		
	2018	2017	2016
	(in thousands, except share and per share data)		
Statement of Operations Data:			
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development[(1)]	41,340	27,899	5,782
General and administrative[(1)]	18,623	7,828	3,486
Total operating expenses	59,963	35,727	9,268
Loss from operations	(59,963)	(35,727)	(9,268)
Other income (expense):			
Interest expense	—	—	(163)
Interest and other income, net	2,448	236	—
Total other income (expense), net	2,448	236	(163)
Net loss	$ (57,515)	$ (35,491)	$ (9,431)
Accretion of redeemable convertible preferred stock to redemption value	(88)	(213)	(107)
Cumulative dividends on redeemable convertible preferred stock	—	(437)	(197)
Reversal of cumulative dividends on redeemable convertible preferred stock	—	634	—
Net loss attributable to common stockholders	$ (57,603)	$ (35,507)	$ (9,735)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.13)	$ (19.12)	$ (65.15)
Weighted average common shares outstanding, basic and diluted	18,389,576	1,856,907	149,414

(1) Amounts include stock-based compensation expense, as follows:

	Year Ended December 31,		
	2018	2017	2016
	(in thousands)		
Research and development	$ 6,032	$1,626	$427
General and administrative	4,671	633	235
	$10,703	$2,259	$662

	As of December 31,		
	2018	2017	2016
		(in thousands)	
Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$142,570	$ 51,402	$ 4,513
Working capital	134,902	48,361	8,534
Total assets	157,313	54,463	11,342
Redeemable convertible preferred stock	—	92,439	17,916
Total stockholders' equity (deficit)	145,648	(42,118)	(8,874)

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes appearing at the end of this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in, or implied by, the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biotechnology company developing novel medicines to extend the curative power of stem cell transplant, gene therapy, genome editing and cell therapy to more patients.

Transplant is a well-established and often curative medical procedure, and emerging data on stem cell gene therapy, which is stem cell transplant using gene-modified stem cells, suggest the potential for meaningful benefit with this newer form of transplant. Stem cell transplant and stem cell gene therapies use the same widely-adopted decades-old transplant process. As it exists today, stem cell transplant is a large market opportunity, and improvements to the current approaches could enable better stem cell transplant and extend stem cell transplant to more patients. The ability to treat patients with a stem cell transplant is limited by the challenges of obtaining sufficient cells to perform the procedure, the inherent morbidity and mortality of current methods used to prepare patients for transplant, and complications following transplant.

At Magenta, we believe we are uniquely positioned to overcome these challenges and to lead a new era in transplant medicine. Our portfolio of product candidates includes biologics, small molecules and a cell therapy designed to address deficiencies in existing approaches and extend the curative power of stem cell transplant, gene therapy, genome editing and cell therapy to more patients across many diseases. Currently, only a fraction of eligible patients with these diseases receive a transplant because the risks and challenges outweigh the potential for a cure. These include diseases where stem cell transplant is a standard of care (e.g., blood cancers such as acute myelogenous leukemia, myelodysplastic syndromes, multiple myeloma, and non-Hodgkin lymphoma), diseases where transplant is performed but limited in use (e.g., hemoglobinopathies such as sickle cell disease and beta-thalassemia), and autoimmune diseases. Emerging clinical data suggest that stem cell transplant may represent a breakthrough approach with curative potential for patients with severe autoimmune diseases. For example, recent results from multiple clinical trials show that patients with autoimmune diseases, including multiple sclerosis and scleroderma, can be cured with a transplant. However, based on our epidemiology analyses, currently only approximately 1 to 2% of eligible patients with multiple sclerosis or scleroderma in the United States and Europe receive a stem cell transplant.

To address the major unmet medical needs in the existing stem cell transplant process, we are developing a stem cell biology discovery platform and comprehensive portfolio of novel therapeutics. Our programs will improve stem cell dose (expansion), stem cell collection (mobilization), patient preparation for transplant (conditioning) and potential post-transplant complications to address key limitations of the stem cell transplant process to allow more patients to benefit. Within our expansion program, MGTA-456 is a cell therapy used with curative intent, and it has the potential to allow more patients to have a better chance for a successful stem cell transplant. We are currently studying it in patients with inherited metabolic disorders and patients with blood cancers. Within our mobilization program, MGTA-145 is focused on enabling physicians to more easily harvest a greater number of blood stem cells, known as hematopoietic stem cells or HSCs, from patients and donors to improve patient outcomes and scale the capacity of transplant and apheresis centers. Our targeted transplant conditioning programs, which prepare the patient for transplant, are designed to selectively remove stem and/or

immune cells from a patient prior to transplant, and to be far less toxic than the decades-old radiation and chemotherapy-based approaches which are still the only available options. Our post-transplant complications program is designed to target the donor immune cells within the patient that cause Graft vs. Host Disease, or GvHD, which can be a fatal complication of transplant.

We intend to become a fully integrated discovery, development and commercial company in the field of transplant medicine. We believe that our product portfolio will offer significant commercial synergies. We are developing our products so that they can each be used individually or in combination with each other. As a result, our portfolio could be utilized in a manner tailored to the patient's disease, such that a patient may receive more than one Magenta therapy as part of their individual transplant journey.

Since our inception in 2015, we have focused substantially all of our efforts and financial resources on organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates, and undertaking preclinical studies, and in the case of MGTA-456, clinical trials. We do not have any products approved for sale and have not generated any revenue from product sales.

On June 25, 2018, we completed an initial public offering, or IPO, of our common stock, pursuant to which we issued and sold 6,666,667 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of $89.9 million after deducting underwriting discounts and commissions and other offering expenses. Prior to the IPO, we funded our operations primarily with proceeds from the sales of redeemable convertible preferred stock and issuance of convertible notes.

Since our inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of our product candidates. Our net loss was $57.5 million for the year ended December 31, 2018 and $35.5 million for the year ended December 31, 2017. As of December 31, 2018, we had an accumulated deficit of $102.7 million. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect our expenses and capital requirements will increase substantially in connection with our ongoing activities, particularly as we:

- continue enrollment in our Phase 2 clinical trial of MGTA-456;
- prepare for and initiate our preclinical studies and clinical trials of our product candidates;
- develop any other future product candidates we may choose to pursue;
- continue research and development and drug discovery and initiate additional clinical trials;
- seek marketing approval for any of our product candidates that successfully complete clinical development, if any;
- maintain compliance with applicable regulatory requirements;
- develop and scale up our capabilities to support our ongoing preclinical activities and clinical trials for our product candidates and commercialization of any of our product candidates for which we obtain marketing approval, if any;
- maintain, expand, protect and enforce our intellectual property portfolio;
- develop and expand our sales, marketing and distribution capabilities for our product candidates for which we obtain marketing approval, if any; and
- expand our operational, financial and management systems and increase personnel, including to support our clinical development and commercialization efforts and our operations as a public company.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for our product candidates. If we obtain regulatory approval for any of our product candidates, we expect to incur significant expenses related to developing our commercialization capability to support product sales, marketing and distribution. Further, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing and distribution or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements as, and when, needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more of our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of December 31, 2018, we had cash, cash equivalents and marketable securities of $142.6 million. We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements through 2020. See "—Liquidity and Capital Resources."

Components of Our Results of Operations

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales. If we enter into license or collaboration agreements for any of our product candidates or intellectual property, we may generate revenue in the future from payments as a result of such license or collaboration agreements. We cannot predict if, when, or to what extent we will generate revenue from the commercialization and sale of our product candidates. We may never succeed in obtaining regulatory approval for any of our product candidates.

Operating Expenses

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which include:

- employee-related expenses, including salaries and related costs, and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with the preclinical and clinical development of our product candidates, including under agreements with contract research organizations, or CROs;
- the cost of consultants and contract manufacturing organizations, or CMOs, that manufacture drug products for use in our preclinical studies and clinical trials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and supplies; and
- payments made under third-party licensing agreements.

We expense research and development costs to operations as incurred. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct research and development expenses are tracked on a program-by-program basis and consist primarily of external costs, such as fees paid to consultants, central laboratories, contractors, CMOs and CROs in connection with our preclinical and clinical development activities. We do not allocate employee costs, costs associated with our platform technology or facility expenses, including depreciation or other indirect costs, to specific product development programs because these costs are deployed across multiple product development programs and, as such, are not separately classified.

The table below summarizes our research and development expenses incurred by development programs:

	Year Ended December 31,		
	2018	2017	2016
		(in thousands)	
Conditioning	$ 9,964	$ 6,259	$ 303
Mobilization	3,665	1,416	431
Expansion	5,465	11,045	13
GvHD	1,070	—	—
Unallocated expenses	21,176	9,179	5,035
Total research and development expenses	$41,340	$27,899	$5,782

The successful development and commercialization of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties, including the following:

- successful completion of preclinical studies and clinical trials;
- receipt and related terms of marketing approvals from applicable regulatory authorities;
- raising additional funds necessary to complete clinical development of and commercialize our product candidates;
- obtaining and maintaining patent, trade secret and other intellectual property protection and regulatory exclusivity for our product candidates;
- making arrangements with third-party manufacturers, or establishing manufacturing capabilities, for both clinical and commercial supplies of our product candidates;
- developing and implementing marketing and reimbursement strategies;
- establishing sales, marketing and distribution capabilities and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- protecting and enforcing our rights in our intellectual property portfolio; and
- maintaining a continued acceptable safety profile of the products following approval.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any of our product candidates.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs, and stock-based compensation, for personnel in executive, finance and administrative functions. General and administrative expenses also include direct and allocated facility-related costs as well as professional fees for legal, patent, consulting, accounting and audit services.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our product candidates. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance, and director and officer insurance costs as well as investor and public relations expenses associated with operating as a public company.

Interest and Other Income, Net

Interest and other income, net, consists of interest income and insignificant amounts of miscellaneous income and expense unrelated to our core operations.

Income Taxes

Since our inception, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in each year or for our earned research and orphan drug tax credits, due to our uncertainty of realizing a benefit from those items. As of December 31, 2018, we had net operating loss carryforwards for federal income tax purposes of $40.6 million, of which $17.5 million begin to expire in 2035 and $23.1 million can be carried forward indefinitely. As of December 31, 2018, we had net operating loss carryforwards for state income tax purposes of $40.8 million which begin to expire in 2035. As of December 31, 2018, we also had available research and orphan drug tax credit carryforwards for federal and state income tax purposes of $3.4 million and $0.8 million, respectively, which begin to expire in 2035 and 2030, respectively.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements included in this Annual Report on Form 10-K, we believe that the following accounting policies are

those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with the preclinical development activities;
- CROs in connection with preclinical and clinical trials;
- CMOs in connection with the production of preclinical and clinical trial materials; and
- investigative sites in connection with clinical trials.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple research institutions and CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation

We measure all stock options and other stock-based awards granted to employees and directors based on the fair value on the date of the grant and recognize compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. Generally, we issue stock options and restricted stock awards, or RSAs, with either service-only vesting conditions and record expense using the straight-line method or service and performance vesting conditions and record expense using the graded-vesting method. We have historically granted stock options with exercise prices equivalent to the fair value of our common stock as of the date of the grant. Prior to our IPO, the estimated fair value of our common stock had been determined by our board of directors as of the date of each award grant, with input from management, considering our most recently available third-party valuations of common stock and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. Subsequent to our IPO, the fair value of our common stock is based on quoted market prices. We estimate the fair value of each stock option award using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions we make for the volatility of our common stock, the expected term of our stock options, the

risk-free interest rate for a period that approximates the expected term of our stock options and our expected dividend yield.

We measure the fair value of stock-based awards granted to non-employees on the date at which the related service is complete. Compensation expense is recognized over the period during which services are rendered by such non-employee consultants until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is re-measured using the then-current fair value of our common stock and updated assumption inputs in the Black-Scholes option-pricing model for options and the then-current fair value of our common stock for restricted stock.

We do not estimate and apply a forfeiture rate as we have elected to account for forfeitures as they occur.

Results of Operations

Comparison of the Years Ended December 31, 2018 and 2017

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

	Year Ended December 31,		
	2018	2017	Change
		(in thousands)	
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	41,340	27,899	13,441
General and administrative	18,623	7,828	10,795
Total operating expenses	59,963	35,727	24,236
Loss from operations	(59,963)	(35,727)	(24,236)
Interest and other income, net	2,448	236	2,212
Net loss	$(57,515)	$(35,491)	$(22,024)

Research and Development Expenses

	Year Ended December 31,		
	2018	2017	Change
		(in thousands)	
Direct research and development expenses by program:			
Conditioning	$ 9,964	$ 6,259	$ 3,705
Mobilization	3,665	1,416	2,249
Expansion	5,465	11,045	(5,580)
GvHD	1,070	—	1,070
Unallocated expenses:			
Personnel related (including stock-based compensation)	10,210	4,384	5,826
Consultant fees	4,000	2,364	1,636
Facility related and other	6,966	2,431	4,535
Total research and development expenses	$41,340	$27,899	$13,441

Expenses related to our conditioning program increased primarily as a result of costs incurred in connection with our collaboration agreement with Heidelberg Pharma Research GmbH for an upfront technology access fee,

research exclusivity fees, target-specific development options and payments for research support related to our drug discovery efforts, primarily lead optimization. Conditioning program costs also increased due to our proof of mechanism and ongoing lead optimization in non-human primate studies. The increase in expense related to our mobilization program was due to an increase in preclinical costs for toxicology studies and manufacturing to support our IND enabling studies. Expenses related to our expansion program decreased primarily as a result of the prior year cost of in-licensing technology of $9.3 million for the rights to MGTA-456 under a license agreement with Novartis. This decrease was partially offset by increased clinical trial and manufacturing costs during the year ended December 31, 2018 incurred for our MGTA-456 Phase 2 study for inherited metabolic diseases, which we initiated in December 2017, and preparation for planned additional clinical trials in indications beyond inherited metabolic diseases. The increase in spending related to our GvHD program was due to costs related to our drug discovery efforts, primarily target validation.

The increase in personnel-related costs included in unallocated expenses was due to an increase in headcount and stock-based compensation expense in our research and development function. Personnel-related costs for the year ended December 31, 2018 and 2017 included stock-based compensation expense of $2.7 million and $0.3 million, respectively. The increase in consultant fees was due to an increase in stock-based compensation issued to nonemployees of $2.0 million, from $1.3 million for the year ended December 31, 2017 to $3.3 million for the year ended December 31, 2018. The increase in facility related and other costs was primarily due to higher facility costs related to the sublease agreement for our Cambridge, Massachusetts facility, which we entered into in May 2018.

General and Administrative Expenses

	Year Ended December 31,		
	2018	2017	Change
		(in thousands)	
Personnel related (including stock-based compensation)	$ 9,809	$3,369	$ 6,440
Professional and consultant fees	5,234	3,217	2,017
Facility related and other	3,580	1,242	2,338
Total general and administrative expenses	$18,623	$7,828	$10,795

The increase in personnel-related costs was primarily a result of an increase in stock-based compensation expense and an increase in headcount. Personnel-related costs for the year ended December 31, 2018 and 2017 included stock-based compensation expense of $4.5 million and $0.6 million, respectively. The increase in professional and consultant fees was primarily due to an increase in patent costs and fees associated with operating as a public company. The increase in facility related and other costs was primarily due to higher facility costs related to the sublease agreement for our Cambridge, Massachusetts facility, which we entered into in May 2018, as well as director and officer insurance costs and an increase in information technology-related expenses.

Interest and Other Income, Net

The increase in interest and other income, net was primarily due to an increase in interest income of $1.9 million resulting from the investment of our net proceeds from our IPO in June 2018 and our sale of series C redeemable convertible preferred stock in April 2018. In addition, the increase was due to $0.4 million of rental income from the sublease of a portion of our Cambridge, Massachusetts facility.

Comparison of the Years Ended December 31, 2017 and 2016

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

	Year Ended December 31,		
	2017	2016	Change
		(in thousands)	
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	27,899	5,782	22,117
General and administrative	7,828	3,486	4,342
Total operating expenses	35,727	9,268	26,459
Loss from operations	(35,727)	(9,268)	(26,459)
Other income (expense):			
Interest expense	—	(163)	163
Interest and other income, net	236	—	236
Total other income (expense), net	236	(163)	399
Net loss	$(35,491)	$(9,431)	$(26,060)

Research and Development Expenses

	Year Ended December 31,		
	2017	2016	Change
		(in thousands)	
Direct research and development expenses by program:			
Conditioning	$ 6,259	$ 303	$ 5,956
Mobilization	1,416	431	985
Expansion	11,045	13	11,032
Unallocated expenses:			
Personnel related (including stock-based compensation)	4,384	1,527	2,857
Consultant fees	2,364	1,442	922
Facility related and other	2,431	2,066	365
Total research and development expenses	$27,899	$5,782	$22,117

Expenses related to our conditioning program increased primarily as a result of our drug discovery efforts for target validation, lead identification and lead optimization. The increase in our mobilization program was due to an increase in preclinical costs for toxicology studies and manufacturing to support our Investigational New Drug, or IND, enabling studies. Expenses related to our expansion program increased primarily due to the cost of in-licensing technology of $9.3 million for the rights to MGTA-456 under a license agreement with Novartis. The cost of in-licensing technology was a result of the issuance of preferred stock to Novartis which was recorded at the fair value of the preferred stock issued. Expansion program costs also increased due to clinical trial costs incurred in preparation for our MGTA-456 Phase 2 study for inherited metabolic diseases, which we initiated in December 2017.

The increase in personnel-related costs included in unallocated expenses was due to an increase in headcount in our research and development function. Personnel-related costs for the years ended December 31, 2017 and 2016 included stock-based compensation expense of $0.3 million and $0.1 million, respectively. The

increase in consultant fees was primarily due to an increase in stock-based compensation from $0.4 million for the year ended December 31, 2016 to $1.3 million for the year ended December 31, 2017. The increase in facility-related and other costs included in unallocated expenses was primarily due to rent expense under our lease agreement for our Cambridge, Massachusetts facility, which we entered into in February 2017.

General and Administrative Expenses

	Year Ended December 31,		
	2017	2016	Change
		(in thousands)	
Personnel related (including stock-based compensation)	$3,369	$1,205	$2,164
Professional and consultant fees	3,217	1,942	1,275
Facility related and other	1,242	339	903
Total general and administrative expenses	$7,828	$3,486	$4,342

The increase in personnel-related costs was primarily a result of an increase in headcount. Personnel-related costs for the years ended December 31, 2017 and 2016 included stock-based compensation expense of $0.6 million and $0.2 million, respectively. The increase in professional and consultant fees was primarily due to an increase in legal, audit and recruiting fees related to ongoing business activities and our preparations to operate as a public company as well as an increase of $0.4 million related to market research activities. The increase in facility-related and other costs was primarily due to rent expense under our lease agreement for our Cambridge, Massachusetts facility, which we entered into in February 2017.

Other Income (Expense)

There was no interest expense for the year ended December 31, 2017. Interest expense was $0.2 million for the year ended December 31, 2016. The decrease of $0.2 million in interest expense was due to the conversion of outstanding convertible notes into redeemable convertible preferred stock in November 2016.

There was $0.2 million other income, net for the year ended December 31, 2017 primarily related to interest income earned on our invested cash balances. There was no other income or expense for the year ended December 31, 2016.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. We have not yet commercialized any of our product candidates and we do not expect to generate revenue from sales of any product candidates for several years, if at all. To date, we have funded our operations primarily with proceeds from the sales of redeemable convertible preferred stock and issuance of convertible notes. On June 25, 2018, we completed the IPO of our common stock, pursuant to which we issued and sold 6,666,667 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of $89.9 million after deducting underwriting discounts and commissions and other offering expenses. As of December 31, 2018, we had cash, cash equivalents and marketable securities of $142.6 million.

Cash Flows

As of December 31, 2018, our principal sources of liquidity were cash, cash equivalents and marketable securities of $142.6 million.

The following table summarizes our sources and uses of cash, cash equivalents and restricted cash for each of the periods presented:

Operating Activities

	Year Ended December 31,		
	2018	2017	2016
		(in thousands)	
Cash used in operating activities	$ (41,886)	$(22,263)	$ (6,529)
Cash used in investing activities	(91,703)	(2,199)	(139)
Cash provided by financing activities	142,147	71,486	10,711
Net increase in cash, cash equivalents and restricted cash	$ 8,558	$ 47,024	$ 4,043

During the year ended December 31, 2018, operating activities used $41.9 million of cash, primarily resulting from our net loss of $57.5 million, partially offset by non-cash charges of $11.5 million and cash provided by changes in our operating assets and liabilities of $4.2 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2018 consisted primarily of a $4.7 million increase in accounts payable and accrued expenses and other current liabilities, and a $1.2 million increase in long-term liabilities, all partially offset by an increase of $1.8 million in prepaid expenses and other current assets.

During the year ended December 31, 2017, operating activities used $22.3 million of cash, primarily resulting from our net loss of $35.5 million, partially offset by non-cash charges of $11.9 million and cash provided by changes in our operating assets and liabilities of $1.3 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2017 consisted primarily of a $2.2 million increase in accounts payable and accrued expenses, partially offset by an increase of $0.9 million in prepaid expenses and other current assets.

During the year ended December 31, 2016, operating activities used $6.5 million of cash, primarily resulting from our net loss of $9.4 million, partially offset by non-cash charges of $1.3 million and cash provided by changes in our operating assets and liabilities of $1.6 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2016 consisted primarily of a $1.6 million increase in accounts payable and accrued expenses.

Changes in accounts payable, accrued expenses and other current liabilities and prepaid expenses in both periods were generally due to growth in our business and the timing of vendor invoicing and payments. The increase in long-term liabilities was related to deferred rent related to our sublease agreement for our Cambridge, Massachusetts facility, which we entered into in May 2018.

Investing Activities

During the year ended December 31, 2018, net cash used in investing activities was primarily attributable to purchases of marketable securities of $84.0 million and purchases of property and equipment of $7.7 million primarily related to leasehold improvements and lab equipment for our Cambridge, Massachusetts facility.

During the years ended December 31, 2017 and 2016, we used $2.2 million and $0.1 million, respectively, to purchase property and equipment.

Financing Activities

During the year ended December 31, 2018, net cash provided by financing activities was $142.1 million, consisting primarily of proceeds from our IPO, net of underwriting discounts and commissions, of $93.0 million

and net proceeds of $52.2 million from the sale of series C redeemable convertible preferred stock, both partially offset by $3.1 million of payments of IPO costs.

During the year ended December 31, 2017, net cash provided by financing activities was $71.5 million, consisting primarily of $6.3 million of the remaining proceeds received in January 2017 from the sale of Series A preferred stock that we recorded as other receivable as of December 31, 2016, $15.4 million of proceeds from the sale of additional shares of Series A preferred stock and $49.8 million of net proceeds received from the sale of Series B preferred stock.

During the year ended December 31, 2016, net cash provided by financing activities was $10.7 million, consisting primarily of proceeds of $6.5 million from the sale of convertible promissory notes and net proceeds of $4.2 million from the sale of Series A preferred stock that we received in 2016.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials for our product candidates in development. In addition, we expect to incur additional costs associated with operating as a public company. The timing and amount of our operating expenditures will depend largely on:

- the initiation, progress, timing, costs and results of current and future preclinical studies and clinical trials for our product candidates;
- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates that we develop or may in-license;
- the terms of any collaboration agreements we may choose to conclude;
- the outcome, timing and cost of meeting and maintaining compliance with regulatory requirements established by the U.S. Food and Drug Administration, the European Medical Agency and other comparable foreign regulatory authorities;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights;
- the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us;
- the effect of existing or new competing technological and market developments;
- the cost and timing of completion of commercial-scale outsourced manufacturing activities; and
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own.

As of December 31, 2018, we had cash, cash equivalents and marketable securities of $142.6 million. We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements through 2020. We have based these estimates on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we expect. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including those listed above.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making acquisitions or capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, limit, reduce or terminate our research, product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

The following summarizes our principal contractual obligations and commitments as of December 31, 2018 and the effects such obligations are expected to have on our liquidity and cash flow in future periods:

	Payments Due By Period				
	Total	Less Than 1 Year	1 to 3 Years	3 to 5 Years	More Than 5 Years
	(in thousands)				
Operating leases[1]	$56,366	$5,434	$11,362	$12,054	$27,516
Contractual obligations[2]	682	682	—	—	—
Total	$57,048	$6,116	$11,362	$12,054	$27,516

(1) In May 2018, we entered into a sublease for up to approximately 69,000 square feet of office and laboratory space in Cambridge, Massachusetts. The term of the lease expires in February 2028.

(2) In August 2018, in connection with the sublease agreement entered into in May 2018 for office and laboratory space in Cambridge, Massachusetts, we entered into an agreement for the build-out and customization of this space. Under the agreement, we are obligated to make payments of up to $6.8 million of which $6.1 million has been incurred through December 31, 2018. The remaining payments are reflected in the table above.

We enter into contracts in the normal course of business with CROs, CMOs and other third parties for clinical trials, preclinical research studies and testing and manufacturing services. These contracts are cancelable by us upon prior written notice. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation. These payments are not included in the preceding table as the amount and timing of such payments are not known.

We have also entered into license and collaboration agreements with third parties, which are in the normal course of business. We have not included future payments under these agreements in the table of contractual obligations above since obligations under these agreements are contingent upon future events such as our achievement of specified development, regulatory, and commercial milestones, or royalties on net product sales. As of December 31, 2018, we were unable to estimate the timing or likelihood of achieving these milestones or generating future product sales.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

Recently Issued and Adopted Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements included in this Annual Report on Form 10-K.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our cash, cash equivalents, and marketable securities as of December 31, 2018 consisted of cash, money market funds and U.S. government securities. We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the investments in our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with and may continue to contract with foreign vendors that are located in Europe. Our operations may be subject to fluctuations in foreign currency exchange rates in the future.

Inflation generally affects us by increasing our cost of labor. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2018, 2017 and 2016.

ITEM 8. CONSOLIDATED FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

MAGENTA THERAPEUTICS, INC.

Index to Consolidated Financial Statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
Magenta Therapeutics, Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Magenta Therapeutics, Inc. and its consolidated subsidiary (the Company) as of December 31, 2018 and 2017, the related consolidated statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the years in the three year period ended December 31, 2018, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the years in the three year period ended December 31, 2018, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

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

Cambridge, Massachusetts
March 19, 2019

MAGENTA THERAPEUTICS, INC.

CONSOLIDATED BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31,	
	2018	**2017**
Assets		
Current assets:		
Cash and cash equivalents	$ 58,345	$ 51,402
Marketable securities	84,225	—
Restricted cash	—	165
Prepaid expenses and other current assets	2,751	936
Total current assets	145,321	52,503
Restricted cash	1,780	—
Property and equipment, net	10,212	1,956
Other assets	—	4
Total assets	$ 157,313	$ 54,463
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 3,492	$ 167
Accrued expenses and other current liabilities	6,927	3,975
Total current liabilities	10,419	4,142
Long-term liabilities	1,246	—
Total liabilities	11,665	4,142
Commitments and contingencies (Note 11)		
Redeemable convertible preferred stock (Series A and B), $0.001 par value; no shares and 49,178,527 shares authorized, issued and outstanding as of December 31, 2018 and 2017, respectively	—	92,439
Stockholders' Equity (Deficit):		
Preferred stock, $0.001 par value; 10,000,000 shares authorized as of December 31, 2018; no shares issued or outstanding	—	—
Common stock, $0.001 par value; 150,000,000 shares and 70,000,000 shares authorized as of December 31, 2018 and 2017, respectively; 34,327,554 shares and 4,458,547 shares issued and 33,305,033 shares and 2,351,247 shares outstanding as of December 31, 2018 and 2017, respectively	33	2
Additional paid-in capital	248,349	3,091
Accumulated other comprehensive loss	(8)	—
Accumulated deficit	(102,726)	(45,211)
Total stockholders' equity (deficit)	145,648	(42,118)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 157,313	$ 54,463

The accompanying notes are an integral part of these consolidated financial statements.

MAGENTA THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	Year Ended December 31,		
	2018	2017	2016
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	41,340	27,899	5,782
General and administrative	18,623	7,828	3,486
Total operating expenses	59,963	35,727	9,268
Loss from operations	(59,963)	(35,727)	(9,268)
Other income (expense):			
Interest expense	—	—	(163)
Interest and other income, net	2,448	236	—
Total other income (expense), net	2,448	236	(163)
Net loss	(57,515)	(35,491)	(9,431)
Accretion of redeemable convertible preferred stock to redemption value	(88)	(213)	(107)
Cumulative dividends on redeemable convertible preferred stock	—	(437)	(197)
Reversal of cumulative dividends on redeemable convertible preferred stock	—	634	—
Net loss attributable to common stockholders	$ (57,603)	$ (35,507)	$ (9,735)
Net loss per share attributable to common stockholders, basic and diluted	$ (3.13)	$ (19.12)	$ (65.15)
Weighted average common shares outstanding, basic and diluted	18,389,576	1,856,907	149,414
Comprehensive loss:			
Net loss	$ (57,515)	$ (35,491)	$ (9,431)
Other comprehensive loss:			
Unrealized loss on marketable securities	(8)	—	—
Total other comprehensive loss	(8)	—	—
Total comprehensive loss	$ (57,523)	$ (35,491)	$ (9,431)

The accompanying notes are an integral part of these consolidated financial statements.

MAGENTA THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)

(In thousands, except share amounts)

	Series A, B and C Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balances at December 31, 2015	—	$ —	—	$—	$ —	$—	$ (289)	$ (289)
Issuance of Series A redeemable convertible preferred stock, net of issuance costs of $107	10,554,990	10,448	—	—	—	—	—	—
Conversion of notes payable and accrued interest into Series A redeemable convertible preferred stock	7,163,984	7,164	—	—	—	—	—	—
Vesting of restricted stock	—	—	773,433	1	12	—	—	13
Issuance of common stock in connection with payment of consultant fees	—	—	189,629	—	157	—	—	157
Issuance of common stock in connection with a license agreement	—	—	385,063	—	318	—	—	318
Accretion of Series A redeemable convertible preferred stock to redemption value	—	107	—	—	(107)	—	—	(107)
Cumulative dividends on redeemable convertible preferred stock	—	197	—	—	(197)	—	—	(197)
Stock-based compensation expense	—	—	—	—	662	—	—	662
Net loss	—	—	—	—	—	—	(9,431)	(9,431)
Balances at December 31, 2016	17,718,974	17,916	1,348,125	1	845	—	(9,720)	(8,874)
Issuance of Series A redeemable convertible preferred stock	15,445,000	15,445	—	—	—	—	—	—
Issuance of Series B redeemable convertible preferred stock, net of issuance costs of $213	12,871,003	49,787	—	—	—	—	—	—
Issuance of Series A and B redeemable convertible preferred stock in connection with a license agreement	3,143,550	9,275	—	—	—	—	—	—
Vesting of restricted stock	—	—	999,253	1	3	—	—	4
Issuance of common stock in connection with payment of consultant fees	—	—	3,869	—	—	—	—	—
Accretion of Series B redeemable convertible preferred stock to redemption value	—	213	—	—	(213)	—	—	(213)
Cumulative dividends on Series A redeemable convertible preferred stock	—	437	—	—	(437)	—	—	(437)
Reversal of Series A redeemable convertible preferred stock dividend	—	(634)	—	—	634	—	—	634
Stock-based compensation expense	—	—	—	—	2,259	—	—	2,259
Net loss	—	—	—	—	—	—	(35,491)	(35,491)
Balances at December 31, 2017	49,178,527	$ 92,439	2,351,247	$ 2	$ 3,091	$—	$ (45,211)	$ (42,118)
Issuance of Series C redeemable convertible preferred stock, net of issuance cost of $88	11,223,102	52,212	—	—	—	—	—	—
Accretion of Series C redeemable convertible preferred stock to redemption value	—	88	—	—	(88)	—	—	(88)
Conversion of preferred stock to common stock	(60,401,629)	(144,739)	23,375,405	23	144,716	—	—	144,739
Issuance of common stock upon initial public offering net of underwriting discounts, commissions and offering costs	—	—	6,666,667	7	89,899	—	—	89,906
Vesting of restricted stock	—	—	907,562	1	(1)	—	—	—
Issuance of common stock upon exercise of stock options	—	—	4,152	—	29	—	—	29
Stock-based compensation expense	—	—	—	—	10,703	—	—	10,703
Unrealized loss on marketable securities	—	—	—	—	—	(8)	—	(8)
Net loss	—	—	—	—	—	—	(57,515)	(57,515)
Balances at December 31, 2018	—	$ —	33,305,033	$ 33	$248,349	$ (8)	$(102,726)	$145,648

The accompanying notes are an integral part of these consolidated financial statements.

MAGENTA THERAPEUTICS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year ended December 31,		
	2018	**2017**	**2016**
Cash flows from operating activities:			
Net loss	$ (57,515)	$(35,491)	$ (9,431)
Adjustments to reconcile net loss to net cash used in operating activities:			
Stock-based compensation expense	10,703	2,259	662
Depreciation and amortization expense	875	376	6
Loss on disposal of property and equipment	67	—	—
Accretion of discounts on marketable securities	(190)	—	—
Non-cash interest expense	—	—	163
License fees paid in common and preferred stock	—	9,275	318
Consultant fees paid in common stock	—	—	157
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(1,811)	(895)	(41)
Accounts payable	2,500	(1,125)	1,006
Accrued expenses and other current liabilities	2,239	3,342	631
Other assets	—	(4)	—
Other long-term liabilities	1,246	—	—
Net cash used in operating activities	(41,886)	(22,263)	(6,529)
Cash flows from investing activities:			
Purchases of property and equipment	(7,672)	(2,199)	(139)
Proceeds from sale of property and equipment	12	—	—
Purchase of marketable securities	(84,043)	—	—
Net cash used in investing activities	(91,703)	(2,199)	(139)
Cash flows from financing activities:			
Proceeds from initial public offering, net of underwriting discounts and commissions	93,000	—	—
Proceeds from issuance of redeemable convertible preferred stock	52,300	71,695	4,305
Proceeds from issuance of convertible notes payable	—	—	6,500
Payments of initial public offering costs	(3,094)	—	—
Payments of redeemable convertible preferred stock issuance costs	(88)	(213)	(107)
Proceeds from issuance of common and restricted stock	—	4	13
Proceeds from exercise of common stock options	29	—	—
Net cash provided by financing activities	142,147	71,486	10,711
Net increase in cash, cash equivalents and restricted cash	8,558	47,024	4,043
Cash, cash equivalents and restricted cash at beginning of period	51,567	4,543	500
Cash, cash equivalents and restricted cash at end of period	$ 60,125	$ 51,567	$ 4,543
Supplemental disclosure of non-cash investing and financing activities:			
Conversion of redeemable convertible preferred stock to common stock	$144,739	$ —	$ —
Purchase of property and equipment included in accounts payable and accrued expenses	$ 1,538	$ —	$ 375
Accretion of redeemable convertible preferred stock to redemption value	$ 88	$ 213	$ 107
Cumulative dividends on Series A redeemable convertible preferred stock	$ —	$ 437	$ 197
Reversal of cumulative dividends on Series A redeemable convertible preferred stock	$ —	$ (634)	$ —
Conversion of notes payable and accrued interest into redeemable convertible preferred stock	$ —	$ —	$ 7,164
Other receivable from investor for redeemable convertible preferred stock	$ —	$ —	$ 6,250

The accompanying notes are an integral part of these consolidated financial statements.

1. Nature of the Business and Basis of Presentation

Magenta Therapeutics, Inc. (the "Company") is a clinical-stage biopharmaceutical company developing novel medicines to bring the curative power of stem cell transplant to more patients. The Company was incorporated under the laws of the State of Delaware in June 2015 as HSCTCo Therapeutics, Inc. In February 2016, the Company changed its name to Magenta Therapeutics, Inc.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

On June 25, 2018, the Company completed its initial public offering ("IPO") pursuant to which it issued and sold 6,666,667 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of $89.9 million, after deducting underwriting discounts and commissions and other offering expenses. Upon the closing of the IPO, the Company's outstanding redeemable convertible preferred stock automatically converted into shares of common stock.

Prior to its IPO, the Company had funded its operations primarily with proceeds from the sales of redeemable convertible preferred stock and issuance of convertible notes. The Company has incurred recurring losses since inception, including net losses of $57.5 million and $35.5 million, respectively, for the years ended December 31, 2018 and 2017. As of December 31, 2018, the Company had an accumulated deficit of $102.7 million. The Company expects to continue to generate operating losses for the foreseeable future. The Company expects that its cash, cash equivalents and marketable securities of $142.6 million as of December 31, 2018 will be sufficient to fund its operating expenses and capital expenditure requirements through at least 12 months from the issuance date of these consolidated financial statements. The future viability of the Company beyond that point is dependent on its ability to raise additional capital to fund its operations.

The Company expects its expenses to increase substantially in connection with ongoing activities, particularly as the Company advances its preclinical activities and clinical trials for its product candidates in development. Accordingly, the Company will need to obtain substantial additional funding in connection with continuing operations. If the Company is unable to raise capital when needed, or on attractive terms, it could be forced to delay, reduce or eliminate its research or drug development programs or any future commercialization efforts. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

The consolidated financial statements include the accounts of the Company and its wholly owned subsidiary. All intercompany balances and transactions have been eliminated.

The consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP").

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these financial statements include, but are not limited to, the accrual for research and development expenses, the valuation of stock-based awards and prior to the IPO, the valuation of common and preferred stock. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

Concentrations of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and marketable securities. The Company maintains all cash, cash equivalents and marketable securities at two accredited financial institutions in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and formulated drugs.

Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less at the date of purchase to be cash equivalents.

Marketable Securities

The Company's marketable securities are classified as available-for-sale and are carried at fair value with the unrealized gains and losses reported as a component of accumulated other comprehensive income (loss) in stockholders' equity. Realized gains and losses and declines in value judged to be other than temporary are included as a component of interest and other income, net based on the specific identification method. The Company classifies its marketable securities with maturities beyond one year as short-term, based on their highly liquid nature and because such marketable securities are available for current operations.

Property and Equipment

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

	Estimated Useful Life
Lab equipment	5 years
Computer equipment	3 years
Furniture and fixtures	5 years
Leasehold improvements	Shorter of life of lease or estimated useful life

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

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value, determined based on discounted cash flows. The Company did not record any impairment losses on long-lived assets during the years ended December 31, 2018, 2017 or 2016.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and marketable securities are carried at fair value, determined according to the fair value hierarchy described above (see Note 3). The carrying values of the Company's accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Classification and Accretion of Redeemable Convertible Preferred Stock

The Company classified redeemable convertible preferred stock outside of stockholders' equity (deficit) because the shares contain certain redemption features that were not solely within the control of the Company. The Company immediately accreted the carrying value of its redeemable convertible preferred stock to redemption value at the end of each reporting period as if the end of the reporting period were the redemption date.

Segment Information

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. All of the Company's tangible assets are held in the United States.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including salaries, stock-based compensation and benefits, facilities costs, depreciation, manufacturing expenses and external costs of outside vendors engaged to conduct preclinical development activities and clinical trials as well as the cost of licensing technology.

Upfront payments and milestone payments made for the licensing of technology are expensed as research and development in the period in which they are incurred. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies both inside and outside of the United States. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies or trials, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Stock-Based Compensation

The Company measures all stock-based awards granted to employees and directors based on the fair value on the date of grant. Compensation expense of those awards is recognized over the requisite service period, which is generally the vesting period of the respective award. Generally, the Company issues awards with either service-only vesting conditions and records the expense using the straight-line method or service and performance vesting conditions and records the expense using the graded-vesting method. The Company accounts for forfeitures as they occur.

The Company measures the fair value of stock-based awards granted to non-employees on the date at which the related service is complete. Compensation expense is recognized over the period during which services are rendered by such non-employee consultants until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of its common stock and updated assumption inputs in the Black-Scholes option-pricing model for options or the then-current fair value of its common stock for restricted stock.

The Company classifies stock-based compensation expense in its statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized

in the consolidated financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in its consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' deficit that result from transactions and economic events other than those with stockholders. For the year ended December 31, 2018, the Company's only element of other comprehensive loss was unrealized gain or (loss) on marketable securities. For the years ended December 31, 2017 and 2016, there was no difference between net loss and comprehensive loss for each of the periods presented in the accompanying consolidated financial statements.

Net Loss per Share

Prior to the closing of its IPO, the Company followed the two-class method when computing net income (loss) per share, as the Company had issued shares that met the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. The Company's redeemable convertible preferred stock contractually entitled the holders of such shares to participate in dividends but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reported a net loss, such losses were not allocated to such participating securities, and as a result, basic and diluted net loss per share were the same.

Subsequent to the closing of its IPO, basic net income (loss) per common share is computed by dividing the net income (loss) by the weighted average number of shares of common stock outstanding for the period. Diluted net income (loss) per common share is computed by dividing net income (loss) by the weighted average number of common shares outstanding for the period, including potential dilutive common shares assuming the dilutive effect of outstanding stock options. For periods in which the Company has reported net losses, diluted net loss per common share is the same as basic net loss per common share, since dilutive common shares are not assumed to have been issued if their affect is anti-dilutive.

In June 2018, upon the closing of the IPO, all outstanding shares of the Company's redeemable convertible preferred stock automatically converted into 23,375,405 shares of the Company's common stock.

The Company reported a net loss attributable to common stockholders for the years ended December 31, 2018, 2017 and 2016.

Recently Adopted Accounting Pronouncements

In May 2017, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2017-09, *Compensation—Stock Compensation* (Topic 718): Scope of Modification Accounting ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. For public and non-public entities, the standard was effective for annual periods beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted. The Company's adoption of ASU 2017-09 on January 1, 2018 did not have an impact on the Company's financial position, results of operations or cash flows.

In November 2016, the FASB issued ASU No. 2016-18, *Statement of Cash Flows (Topic 230)* ("ASU 2016-18"), which requires that amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. For public entities, ASU 2016-18 is effective for fiscal years beginning after December 15, 2017 and interim periods within those fiscal years. For non-public entities and emerging growth companies that choose to take advantage of the extended transition period, the standard is effective for fiscal years beginning after December 15, 2018. Early adoption is permitted for all entities. As early adoption was permitted, the Company adopted this standard retrospectively as of December 31, 2018. Restricted cash is now included as a component of cash, cash equivalents and restricted cash on the Company's consolidated statement of cash flows. Upon the adoption of ASU 2016-18, the amount of cash and cash equivalents previously presented on the consolidated statements of cash flows for the year ended December 31, 2017 increased by less than $0.1 million as of beginning of the year and by $0.1 million as of end of the year and for the year ended December 31, 2016 increased by less than $0.1 as of the end of the year to reflect the inclusion of restricted cash in the amount reported for changes in cash, cash equivalents and restricted cash.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, *Leases* ("ASU 2016-02"). ASU 2016-02 will require lessees to recognize most leases on their balance sheet as a right-of-use asset and a lease liability. Leases will be classified as either operating or finance, and classification will be based on criteria similar to current lease accounting, but without explicit bright lines. For public entities, the guidance is effective for annual reporting periods beginning after December 15, 2018 and for interim periods within those fiscal years. For non-public entities and emerging growth companies that choose to take advantage of the extended transition period, the guidance is effective for annual reporting periods beginning after December 15, 2019. Early adoption is permitted for all entities. ASU 2016-02 initially required adoption using a modified retrospective approach, under which all years presented in the financial statements would be prepared under the revised guidance. In July 2018, the FASB issued ASU No. 2018-11, Leases (Topic 842), which added an optional transition method under which financial statements may be prepared under the revised guidance for the year of adoption, but not for prior years. Under the latter method, entities will recognize a cumulative catch-up adjustment to the opening balance of retained earnings in the period of adoption. The Company is currently evaluating the impact that the adoption of ASU 2016-02 will have on its financial statements.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"). This ASU is intended to simplify aspects of share-based compensation issued to non-employees by making the guidance consistent with the accounting for employee share-based compensation. For public entities, ASU 2018-07 is required to be adopted for annual periods beginning after December 15, 2018, including interim periods within those fiscal years. For non-public entities and emerging growth companies that choose to take advantage of the extended transition period, ASU 2018-07 is effective for annual periods beginning after December 15, 2019. Early adoption is permitted for all entities but no earlier than the Company's adoption of ASU 2014-09. The Company

is currently evaluating whether to early adopt ASU 2018-07 and the impact that the adoption will have on its financial statements.

3. Marketable Securities and Fair Value Measurements

As of December 31, 2018, marketable securities by security type consisted of (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
United States Treasury Notes (due within one year)	$71,944	$5	$ (9)	$71,940
Agency Bonds (due within one year)	12,289	1	(5)	12,285
Total	$84,233	$6	$(14)	$84,225

The Company had no marketable securities as of December 31, 2017.

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	Fair Value Measurements at December 31, 2018 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$45,725	$ —	$—	$ 45,725
United States Treasury Notes	—	12,488	—	12,488
Marketable securities:				
United States Treasury Notes	—	71,940	—	71,940
Agency Bonds	—	12,285	—	12,285
Total	$45,725	$96,713	$—	$142,438

	Fair Value Measurements at December 31, 2017 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$51,147	$ —	$—	$ 51,147
Total	$51,147	$ —	$—	$ 51,147

During the years ended December 31, 2018 and 2017, there were no transfers between Level 1, Level 2 and Level 3.

4. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

	December 31,	
	2018	2017
Laboratory and computer equipment	$ 4,160	$2,120
Furniture and fixtures	743	77
Leasehold improvements	6,399	141
	11,302	2,338
Less: Accumulated depreciation and amortization	(1,090)	(382)
	$10,212	$1,956

Depreciation and amortization expense was $0.9 million, $0.4 million and less than $0.1 million for the years ended December 31, 2018, 2017 and 2016, respectively.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31,	
	2018	2017
Accrued external research and development expenses	$2,619	$1,531
Accrued payroll and related expenses	2,526	1,330
Accrued professional fees	787	963
Accrued leasehold improvements	501	—
Accrued other	494	151
	$6,927	$3,975

6. Convertible Promissory Notes

In December 2015, the Company issued convertible promissory notes in the aggregate principal amount of $0.5 million. In 2016, the Company issued convertible promissory notes in the amount of $6.5 million. The notes accrued interest at an annual rate of 6% payable upon maturity at one year after issuance. In November 2016, the total outstanding principal and accrued interest on the notes of $7.2 million converted into 7,163,984 shares of the Company's Series A redeemable convertible preferred stock (the "Series A Preferred Stock") at a price of $1.00 per share. The convertible promissory notes did not contain any features which were required to be bifurcated and accounted for separately.

7. Redeemable Convertible Preferred Stock

In June 2018, the Company effected a one-for-2.58398 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company's redeemable convertible preferred stock ("Preferred Stock"). Accordingly, all share and per share amounts for all periods presented in the accompanying consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios.

As of December 31, 2017, the Company's certificate of incorporation, as amended and restated (the "Certificate of Incorporation"), authorized the Company to issue 49,178,527 shares of $0.001 par value

preferred stock, of which 35,663,974 shares were designated as Series A Preferred Stock and 13,514,553 shares were designated as Series B redeemable convertible preferred stock (the "Series B Preferred Stock"). In April 2018, in connection with the sale of Series C redeemable convertible preferred stock (the "Series C Preferred Stock"), the Company's Certificate of Incorporation was amended and restated to authorize the Company to issue 11,226,000 shares of Series C Preferred Stock. The Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock are collectively referred to as the "Preferred Stock".

Series A Preferred Stock Purchase Agreement

In November 2016, the Company entered into a Series A Preferred Stock purchase agreement and issued 17,718,974 shares of Series A Preferred Stock at $1.00 per share for gross proceeds of $10.6 million and the conversion of principal and accrued interest of $7.2 million on convertible promissory notes (see Note 6). Cash proceeds of $6.3 million were received in January 2017. The Company incurred issuance costs of $0.1 million in connection with the issuance and sale of the Series A Preferred Stock.

The Series A Preferred Stock purchase agreement obligated the Company to sell and the Purchasers (as defined) to purchase at $1.00 per share an aggregate of 15,445,000 shares of Series A Preferred Stock at a subsequent closing (the "First Subsequent Closing") and at the Company's sole discretion an aggregate of 15,500,000 shares of Series A Preferred Stock at a second subsequent closing (the "Second Subsequent Closing"), upon the achievement or waiver of certain milestones. In April 2017, in connection with the First Subsequent Closing and the waiver of the applicable milestones, the Company received gross proceeds of $15.4 million for the issuance and sale of 15,445,000 shares of Series A Preferred Stock. The Company determined that the future tranche obligations of the Series A Preferred Stock purchase agreement did not meet the definition of a freestanding financial instrument because, while separately exercisable, it was not legally detachable. Further, the Company determined that the embedded future tranche obligation did not require bifurcation for accounting purposes as it was clearly and closely related to the economic characteristics and risks of the initial preferred shares and would not meet the definition of a derivative on a standalone basis. There was no Second Subsequent Closing.

Series B Preferred Stock Purchase Agreement

In April 2017, the Company entered into a Series B Preferred Stock purchase agreement and issued and sold 12,871,003 shares of Series B Preferred Stock at $3.8847 per share for gross proceeds of $50.0 million in two separate closings. The Company incurred issuance costs of $0.2 million in connection with the issuance and sale of the Series B Preferred Stock.

Series C Preferred Stock Purchase Agreement

In April 2018, the Company entered into a Series C Preferred Stock purchase agreement, pursuant to which the Company issued and sold 11,223,102 shares of Series C redeemable convertible Preferred Stock at a price of $4.66 per share for total gross proceeds of $52.3 million.

Issuance of Preferred Stock for License

In consideration for the grant of rights to the Company pursuant to a license agreement between the Company and Novartis International Pharmaceutical Ltd., ("Novartis"), dated April 3, 2017 (see Note 11), the Company issued 2,500,000 shares of Series A Preferred Stock and 643,550 shares of Series B Preferred Stock to Novartis.

Upon issuance of each class of Preferred Stock the Company assessed the embedded conversion and liquidation features of the securities and determined that such features did not require the Company to separately account for these features. The Company also concluded that no beneficial conversion feature existed upon the

issuance date of each class of Preferred Stock. As of December 31, 2017, the Preferred Stock consisted of the following (in thousands, except share amounts):

	December 31, 2017				
	Preferred Stock Authorized	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A Preferred Stock	35,663,974	35,663,974	$39,939	$35,664	13,801,936
Series B Preferred Stock	13,514,553	13,514,553	52,500	52,500	5,230,130
	49,178,527	49,178,527	$92,439	$88,164	19,032,066

Upon closing of the IPO in June 2018, all of the outstanding shares of Preferred Stock, including the shares of Series C Preferred Stock issued in April 2018, were converted into 23,375,405 shares of common stock. Prior to the conversion, the holders of the Preferred Stock had the following rights and preferences:

Voting Rights

The holders of Preferred Stock were entitled to vote, together with the holders of common stock, on all matters submitted to stockholders for a vote. Each preferred stockholder was entitled to the number of votes equal to the number of shares of common stock into which each Preferred Stock was convertible at the time of such vote.

Dividends

There were no stated dividends on the Preferred Stock.

At the time of issuance, the holders of Series A Preferred Stock were entitled to receive cumulative dividends at an annual rate of 8% of the Original Issue Price (as described below). However, in connection with the Series B Preferred Stock financing in April 2017, holders of Series A Preferred Stock waived their right to dividends. During the years ended December 31, 2017 and 2016, the Company accrued dividends of $0.4 million and $0.2 million, respectively, and recorded an increase to the carrying value of the Series A Preferred Stock with an offset to additional paid in capital. In April 2017, in connection with the elimination of dividends on the Series A Preferred Stock, the Company reduced the carrying value of the Series A Preferred Stock by $0.6 million with an offset to additional paid in capital.

The Company may not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Company unless the holders of Preferred Stock then outstanding shall first receive, or simultaneously receive, dividends on each outstanding share of Preferred Stock. To date, no dividends had been approved or paid.

Liquidation

In the event of any liquidation, dissolution or winding up of the Company or Liquidating Event (as described below), the holders of Preferred Stock then outstanding were entitled, on a pari passu basis, to be paid out of the assets of the Company prior to any payments made to the holders of common stock by reason of their ownership thereof, an amount per share equal to the greater of the Original Issue Price (as described below) plus any dividends declared but unpaid or such amount per share as would have been payable had all shares of Preferred Stock been converted into common stock. If upon liquidation the assets of the Company available for distribution to its stockholders were insufficient to pay the holders of Preferred Stock the full amount, the holders of the shares of Preferred Stock would share ratably in any distribution in proportion to the respective amount of Preferred Stock.

Upon completion of the liquidation preference distribution to the holders of Preferred Stock, the remaining assets of the Company would be distributed among holders of common stock, pro rata, based on the number of shares held by each such holder. Unless the holders of a majority of the outstanding Preferred Stock, voting together as a single class, and in certain circumstances, holders of a majority of the outstanding Series B Preferred Stock, voting as a separate class, elect otherwise, Liquidating Event would include a merger or consolidation (other than one in which shareholders of the Company own a majority of the voting power of the outstanding shares of the surviving or acquiring corporation) or a sale, lease, transfer, exclusive license or other disposition of all or substantially all of the assets of the Company.

Conversion

Each share of Preferred Stock was convertible at the option of the stockholder at any time without the payment of additional consideration by the holder thereof, into a number of shares of common stock at the applicable conversion ratio determined by dividing the Original Issue Price by the Conversion Price of each series. The Original Issue Price was $1.00 per share for Series A Preferred Stock, $3.8847 per share for Series B Preferred Stock and $4.66 per share for Series C Preferred Stock. The Conversion Price is $2.58398 per share for Series A Preferred Stock, $10.03799 per share for Series B Preferred Stock and $12.04135 per share for Series C Preferred Stock, subject to appropriate adjustment in the event of any stock split, stock dividend, combination or other similar recapitalization and other adjustments as set forth in the Company's certificate of incorporation, as amended and restated.

Each share of Preferred Stock automatically converts into shares of common stock at the then effective conversion ratio (i) upon the closing of an initial public offering with proceeds to the Company of at least $50.0 million, after deducting underwriting discounts and commissions or (ii) upon the vote or written consent of the holders of a majority of the then outstanding shares of the Preferred Stock, voting together as a single class, and holders of a majority of the outstanding Series B Preferred Stock, voting as a separate class.

As of December 31, 2017, all outstanding shares of Preferred Stock were convertible into shares of common stock on a 2.58398-for-one basis.

Redemption Rights

On or after the fifth anniversary of the Series C original issue date, or April 2, 2023, shares of the Preferred Stock were subject to mandatory redemption by the Company in three equal annual installments beginning 60 days after receipt of a notice of redemption from the holders of a majority of the voting power of the holders of the outstanding Preferred Stock, voting as a single class. As of December 31, 2017, the redemption price for the Preferred Stock was equal to the Original Issuance Price per share, plus any accruing dividends accrued but unpaid therein.

Reissuance

Shares of any Preferred Stock that were redeemed or converted will be retired or canceled and may not be reissued by the Company.

8. Common Stock

On June 6, 2018, the Company effected a one-for-2.58398 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of the Company's Preferred Stock. Accordingly, all share and per share amounts for all periods presented in the accompanying consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the preferred stock conversion ratios.

On June 25, 2018, the Company completed its IPO pursuant to which it issued and sold 6,666,667 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of $89.9 million, after deducting underwriting discounts and commissions and other offering expenses. Upon the closing of the IPO, the Company's outstanding redeemable convertible preferred stock automatically converted into shares of common stock.

In 2017 and 2016, the Company issued 218,654 and 4,377,280 shares, respectively, of restricted stock to employees and consultants of the Company. Unvested shares of restricted stock may not be sold or transferred by the holder. These restrictions lapse according to the vesting conditions of each award (see Note 9). The Company issued 193,498 shares of common stock to two investors in exchange for consulting services (see Note 14). In 2016, the Company issued 385,063 shares of common stock in connection with a license agreement (see Note 11).

As of December 31, 2018, the Company's amended and restated certificate of incorporation authorizes the Company to issue 150,000,000 shares of common stock, par value $0.001 per share, and 10,000,000 shares of preferred stock, par value $0.001 per share.

As of December 31, 2017, the Company's Certificate of Incorporation authorized the Company to issue 70,000,000 shares of common stock, $0.001 par value. In connection with the Series C Preferred Stock purchase agreement, the Company's Certificate of Incorporation was amended and restated to increase the number of common stock authorized from 70,000,000 to 85,000,000 shares.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are not entitled to receive dividends unless declared by the board of directors.

9. Stock-Based Compensation

2016 Stock Option and Grant Plan

In April 2018, the Company's 2016 Stock Option and Grant Plan (the "2016 Plan") was amended to increase the number of shares reserved for issuance under the 2016 Plan by 1,884,850 shares to 5,900,917 shares. Upon effectiveness of the Company's 2018 Stock Option and Incentive Plan, (the "2018 Plan") in June 2018, the remaining 1,142,136 shares available under the 2016 Plan became available for issuance under the 2018 Plan and no future issuance will be made under the 2016 Plan. Additionally, shares of common stock underlying any awards under the 2016 Plan that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without any issuance of stock, expire or are otherwise terminated (other than by exercise) will be available for future awards under the 2018 Plan.

2018 Stock Option and Incentive Plan

The 2018 Plan was approved in May 2018 and became effective on June 19, 2018. The 2018 Plan provides for the grant of incentive stock options, non-statutory stock options, restricted stock, restricted stock units, stock appreciation rights, performance units and performance shares to employees, directors and consultants. The Company initially reserved 1,765,162 shares of common stock plus the 1,142,136 shares of common stock remaining available for issuance under the 2016 Plan for the issuance of awards under the 2018 Plan. As of December 31, 2018, 2,693,929 shares remained available for future grants under the 2018 Plan.

The 2018 Plan provides that the number of shares reserved and available for issuance under the 2018 Plan will automatically increase each January 1, beginning on January 1, 2019, by 4% of the outstanding number of shares of the Company's common stock on the immediately preceding December 31 or such lesser number of shares as determined by the Company's compensation committee. This number is subject to adjustment in the event of a stock split, stock dividend or other change in capitalization. The number of shares reserved for issuance under the 2018 Plan was increased by 1,332,201 shares effective January 1, 2019.

Both the 2016 Plan and the 2018 Plan are administered by the board of directors, or at the discretion of the board of directors, by a committee of the board. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or their committee if so delegated, except that the term of awards may not be greater than ten years. Vesting periods are determined at the discretion of the board of directors. Awards granted to employees and directors typically vest over three or four years. The exercise price for stock options granted may not be less than the fair value of common stock as of the date of grant. Subsequent to the IPO, the fair value of common stock is based on quoted market prices. Prior to the IPO, the Company's board of directors valued the Company's common stock, taking into consideration its most recently available valuation of common stock performed by third parties as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant.

Common Stock Option Valuation

The fair value of stock option grants is estimated using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, the Company estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

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

	Year Ended December 31,	
	2018	**2017**
Risk-free interest rate	2.8%	2.0%
Expected term (in years)	6.2	6.0
Expected volatility	78.4%	78.1%
Expected dividend yield	0%	0%

There were no common stock options granted during the year ended December 31, 2016.

Common Stock Option Activity

The following table summarizes the Company's option activity since December 31, 2017:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term	Aggregate Intrinsic Value
			(in years)	(in thousands)
Outstanding as of December 31, 2017	624,406	$4.84	9.8	$1,791
Granted	3,123,026	9.40		
Exercised	(4,152)	7.71		
Forfeited	(233,451)	8.29		
Outstanding as of December 31, 2018	3,509,829	$8.67	9.2	$ 526
Options vested and expected to vest as of December 31, 2018	3,509,829	$8.67	9.2	$ 526
Options exercisable as of December 31, 2018	573,669	$7.51	9.1	$ 179

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of options exercised during the year ended December 31, 2018 was less than $0.1 million. There were no options exercised during the years ended December 31, 2017 and 2016.

The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2018 and 2017 was $6.56 and $3.31, respectively.

As of December 31, 2018, there was 187,686 stock options held by non-employees. There were no stock options held by non-employees as of December 31, 2017.

Restricted Stock Activity

Unvested shares of restricted stock may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award.

The table below summarizes the Company's restricted stock activity for grants issued under the 2016 Plan since December 31, 2017:

	Number of Shares	Weighted Average Grant Date Fair Value
Outstanding as of December 31, 2017	2,107,300	$1.25
Vested	(907,562)	1.23
Forfeited	(177,217)	1.89
Outstanding as of December 31, 2018	1,022,521	$1.16

The total fair value of restricted stock vested during the years ended December 31, 2018, 2017 and 2016 was $9.3 million, $1.9 million and $0.6 million, respectively.

As of December 31, 2018 and 2017, there were 326,017 and 626,376, respectively, of unvested shares of restricted stock held by non-employees.

Stock-Based Compensation

Stock-based compensation expense was classified in the statements of operations and comprehensive loss as follows (in thousands):

	Year Ended December 31,		
	2018	2017	2016
Research and development expenses	$ 6,032	$1,626	$427
General and administrative expenses	4,671	633	235
	$10,703	$2,259	$662

As of December 31, 2018, total unrecognized compensation cost related to the unvested stock-based awards was $16.8 million, which is expected to be recognized over a weighted average period of 2.3 years.

10. Net Loss per Share

Net Loss per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Year Ended December 31,		
	2018	2017	2016
Numerator:			
Net loss	$ (57,515)	$ (35,491)	$ (9,431)
Accretion of redeemable convertible preferred stock to redemption value	(88)	(213)	(107)
Cumulative dividends on redeemable convertible preferred stock	—	(437)	(197)
Reversal of cumulative dividends on redeemable convertible preferred stock	—	634	—
Net loss attributable to common stockholders	$ (57,603)	$ (35,507)	$ (9,735)
Denominator:			
Weighted average common shares outstanding, basic and diluted	18,389,576	1,856,907	149,414
Net loss per share attributable to common stockholders, basic and diluted	$ (3.13)	$ (19.12)	$ (65.15)

Common Stock Equivalents

The following common stock equivalents presented based on amounts outstanding at each period end, have been excluded from the calculation of diluted net loss per share because including them would have had an anti-dilutive impact:

	December 31,		
	2018	2017	2016
Stock options to purchase common stock	3,509,829	624,406	—
Unvested restricted common stock	1,022,521	2,107,300	3,603,847
Redeemable convertible preferred stock (as converted to common stock)	—	19,032,066	6,857,241
	4,532,350	21,763,772	10,461,088

11. Commitments and Contingencies

Leases

In May 2018, the Company entered into a sublease for up to approximately 69,000 square feet of office and laboratory space in Cambridge, Massachusetts. The sublease is subject and subordinate to a prime lease between the sublandlord and the prime landlord. The term of the sublease commenced in June 2018 and expires in February 2028. The sublandlord has the right to terminate the sublease after five years. The Company is also obligated to pay real estate taxes and other costs related to the premises, including costs of operations and management of the new leased premises. In connection with the sublease, the sublandlord agreed to fund up to $0.7 million in tenant improvements to the leased facility which was included in prepaid expenses and other current assets at December 31, 2018. The Company is required to maintain a cash balance of $1.8 million to secure a letter of credit associated with the sublease. This amount was classified as noncurrent restricted cash in the balance sheet at December 31, 2018.

The tenant improvement allowance and payment escalations specified in the lease agreement are accrued or deferred as appropriate such that rent expense per square foot is recognized on a straight-line basis over the terms of occupancy. The Company recorded rent expense of $4.4 million, $1.1 million and less than $0.1 million during the years ended December 31, 2018, 2017 and 2016, respectively.

In the fourth quarter of 2018, with the landlord's consent, the Company entered into two two-year sub-subleases of approximately 27,000 square feet of office space in Cambridge, Massachusetts under which it will receive $4.5 million of payments over the sub-sublease term.

In August 2018, in connection with the sublease agreement entered into in May 2018, the Company entered into an agreement for the build-out and customization of this space. Under the agreement, the Company is obligated to make payments of up to $6.8 million, of which $6.1 million has been incurred through December 31, 2018.

The Company had a sublease for office space in Cambridge, Massachusetts that expired in September 2018. The Company was required to maintain a cash balance of $0.2 million to secure a letter of credit associated with this sublease. This amount was classified as restricted cash in the balance sheet at December 31, 2017.

As of December 31, 2018, the future minimum lease payments due under the noncancelable operating lease is as follows (in thousands):

2019	$ 5,434
2020	5,597
2021	5,765
2022	5,938
2023	6,116
Thereafter	27,516
	$56,366

Collaboration Agreement

In March 2018, the Company entered into a collaboration agreement with Heidelberg Pharma Research GmbH, ("HDPR") whereby the parties agreed to combine the Company's stem cell platform with proprietary antibodies across up to four exclusive targets with HDPR's proprietary Antibody Targeted Amanitin Conjugates platform. Under the agreement, the Company may pay upfront technology access fees, research exclusivity fees and payment for research support. Additionally, upon the exercise of certain license rights, the Company may be obligated to pay HDPR development, regulatory and commercial milestone payments of up to $83.5 million per

target as well as royalties on net sales of products licensed under the agreement. During the year ended December 31, 2018, the Company recorded $1.7 million of research and development expense related to this agreement for upfront technology access fees, research exclusivity fees and research support.

Intellectual Property Licenses

In November 2016, the Company entered into a license agreement with the President and Fellows of Harvard College ("Harvard") for an exclusive, worldwide, royalty-bearing license for certain technologies related to conditioning and mobilization. In consideration for these rights the Company paid Harvard an upfront fee of $0.1 million and reimbursed Harvard $0.3 million for costs incurred by Harvard related to the patented technology, which amounts were recorded as research and development expense during the year ended December 31, 2016. The Company also issued 385,063 shares of common stock in connection with the license agreement. The fair value of the shares of $0.3 million as of the issuance date was recorded as research and development expense during the year ended December 31, 2016. The Company is obligated to pay Harvard maintenance fees of less than $0.1 million annually through 2019 and $0.1 million annually thereafter and to reimburse qualified expenses related to the patents. The Company is also obligated to pay milestone payments of up to $7.4 million for the first two licensed products upon the achievement of certain development and regulatory milestones and to pay royalties on a product-by-product and country-by-country basis on net sales of products licensed under the agreement. As of December 31, 2018, no milestones related to this agreement have been met.

In April 2017, the Company entered into a license agreement with Novartis International Pharmaceutical Ltd. ("Novartis") to use and develop certain patent rights (the "Novartis License"). Under the Novartis License, the Company was granted an exclusive, worldwide, sublicensable license to research, develop and commercialize certain licensed products that contain Novartis compounds for the expansion of cord blood derived non-gene-edited/-modified hematopoietic stem cells. In consideration for these rights, the Company issued 2,500,000 shares of Series A redeemable convertible preferred stock and 643,550 shares of Series B redeemable convertible preferred stock to Novartis. The total fair value of the shares of $9.3 million as of the issuance date was recorded as research and development expense during the year ended December 31, 2017. The Company is obligated to make payments of up to $177.0 million upon the achievement of specified clinical and regulatory milestones and up to $125.0 million upon the achievement of specified commercial milestones and to pay tiered royalties, on a product-by-product and country-by-country basis, up to a maximum of 20% on net sales of products licensed under the agreement. As of December 31, 2018, no milestones related to the Novartis License have been met.

The Company has agreements with third parties in the normal course of business, under which it can license certain developed technologies. If the Company exercises its rights to license the respective technologies, it may be subject to additional fees and milestone payments. During the year ended December 31, 2018, the Company expensed $0.8 million related to the license of certain developed technologies under these agreements.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and senior management that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. The Company is not aware of any claims under indemnification arrangements, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2018 or 2017.

Legal Proceedings

The Company is not currently a party to any material legal proceedings. At each reporting date, the Company evaluates whether or not a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company expenses the costs related to its legal proceedings as they are incurred.

12. 401(k) Savings Plan

The Company has a 401(k) available for participating employees who meet certain eligibility requirements. Eligible employees may defer a portion of their salary as defined by the plan. Company contributions to the plan may be made at the discretion of the Board of Directors of the Company. To date, the Company has not made any contributions to the plan.

13. Income Taxes

During the years ended December 31, 2018, 2017 and 2016, the Company recorded no income tax benefits for the net operating losses incurred or for the research and orphan drug tax credits generated in each year, due to its uncertainty of realizing a benefit from those items.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,		
	2018	2017	2016
Federal statutory income tax rate	21.0%	34.0%	34.0%
State taxes, net of federal benefit	5.6	4.9	4.6
Research and orphan drug tax credits	4.7	3.4	1.8
Impact of 2018 tax rate change on temporary differences	—	(13.4)	—
Other	(2.1)	(3.7)	(4.2)
Increase in deferred tax asset valuation allowance	(29.2)	(25.2)	(36.2)
Effective income tax rate	— %	— %	— %

Net deferred tax assets as of December 31, 2018 and 2017 consisted of the following (in thousands):

	December 31,	
	2018	2017
Deferred tax assets:		
Net operating loss carryforwards	$ 11,106	$ 5,158
Capitalized research and development expenses	12,030	5,925
Research and orphan drug tax credit carryforwards	4,108	1,400
Stock compensation expense	1,336	9
Accrued expense	664	20
Other	360	—
Total deferred tax assets	29,604	12,512
Valuation allowance	(29,243)	(12,466)
Net deferred tax assets	361	46
Deferred tax liabilities:		
Depreciation and amortization	(361)	(46)
Total deferred tax liabilities	(361)	(46)
Net deferred tax assets and liabilities	$ —	$ —

On December 22, 2017, the Tax Cuts and Jobs Act (the "TCJA") was signed into United States law. The TCJA included a number of changes to existing tax law, including, among other things, a permanent reduction in the federal corporate income tax rate from 35% to 21%, effective as of January 1, 2018, as well as limitation of the deduction for net operating losses to 80% of annual taxable income and elimination of net operating loss carrybacks, in each case, for losses arising in taxable years beginning after December 31, 2017 (though any such net operating losses may be carried forward indefinitely). The tax rate change resulted in (i) a reduction in the gross amount of the Company's deferred tax assets recorded as of December 31, 2017, without an impact on the net amount of its deferred tax assets, which are recorded with a full valuation allowance, and (ii) no income tax expense or benefit being recognized as of the enactment date of the TCJA. In connection with the TCJA, the Company remeasured its deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future, which is generally 21% for federal tax purposes. The remeasurement of the Company's deferred tax assets and liabilities resulted in a provision of $4.8 million to income tax expense which was offset by a corresponding decrease in the valuation allowance.

As of December 31, 2018, the Company had net operating loss carryforwards for federal income tax purposes of $40.6 million, of which $17.5 million begin to expire in 2035 and $23.1 million can be carried forward indefinitely. As of December 31, 2018, the Company had net operating loss carryforwards for state income tax purposes of $40.8 million which begin to expire in 2035. As of December 31, 2018, the Company also had available research and orphan drug tax credit carryforwards for federal and state income tax purposes of $3.4 million and $0.8 million, respectively, which begin to expire in 2035 and 2030, respectively. Utilization of the net operating loss carryforwards and research and orphan drug tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended (the "Code") due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. The Company has not conducted a formal study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382 and 383 of the Code, at any time since inception, utilization of the net operating loss carryforwards or research and orphan drug tax credit carryforwards may be subject to an annual limitation under Section 382 and 383 of the Code, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and orphan drug tax credit carryforwards before utilization.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. The Company considered its history of cumulative net losses incurred since inception and its lack of commercialization of any products since inception and has concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2018 and 2017. The Company reevaluates the positive and negative evidence at each reporting period.

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2018, 2017 and 2016 related primarily to the increase in net operating loss carryforwards and research and orphan drug tax credit carryforwards, partially offset in 2017 by a decrease in deferred tax assets resulting from the decreased federal corporate tax rate, and were as follows (in thousands):

	Year Ended December 31,		
	2018	2017	2016
Valuation allowance as of beginning of year	$12,466	$ 3,540	$ 129
Net increases recorded to income tax provision	16,777	8,926	3,411
Valuation allowance as of end of year	$29,243	$12,466	$3,540

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2018 or 2017.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's tax years are open under statute from 2015, the inception of the Company, to the present. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision.

14. Related Parties

Atlas Venture Life Science Advisors, LLC and Third Rock Ventures LLC

During the years ended December 31, 2017 and 2016, the Company received consulting, advisory and related services from Atlas Venture Life Science Advisors, LLC and Third Rock Ventures LLC, holders, together with their affiliates, of more than 5% of the Company's common stock outstanding. For the years ended December 31, 2017 and 2016, the Company recorded $0.2 million and $1.1 million, respectively, of general and administrative expenses for management and advisory services and other related services provided by these investors. The Company also issued 96,749 shares of common stock to each investor and recorded $0.2 million of general and administrative expense for the year ended December 31, 2016 related to these shares. There were no services provided during the year ended December 31, 2018 and no amounts owed to these investors as of December 31, 2018 or 2017.

Be The Match BioTherapies, LLC

Effective March 2018, the President of Be The Match BioTherapies, LLC became a member of the Company's board of directors. The Company has a collaboration agreement with Be The Match BioTherapies, LLC and a research agreement with an affiliated organization, Center for International Blood and Marrow Transplant Research. For the year ended December 31, 2018, the Company recorded $0.6 million to research and development expenses and $0.2 million to general and administrative expenses and made payments of $0.6 million related to these agreements. As of December 31, 2018, amounts on the balance sheet related to these agreements was $0.4 million included in accounts payable and accrued expenses and other current liabilities.

In April 2018, the Company sold 246,781 shares of Series C Preferred Stock to Be The Match Bio Therapies, LLC for $1.1 million (see Note 7).

Novartis

In April 2017, the Company entered into a license agreement with Novartis, a beneficial owner of 5% or more of its outstanding shares of capital stock as of December 31, 2017, to use and develop certain patent rights. In consideration for these rights, the Company issued 2,500,000 shares of Series A Preferred Stock and 643,550 shares of Series B Preferred Stock to Novartis. The fair value of the shares of $9.3 million as of the issuance date was recorded as research and development expense during the year ended December 31, 2017 (see Note 11). As of December 31, 2018, Novartis is no longer an owner of 5% or more of the Company's outstanding stock.

15. Selected Quarterly Financial Data (Unaudited)

The following information has been derived from unaudited consolidated financial statements that, in the opinion of management, include all recurring adjustments necessary for a fair statement of such information (in thousands except per share data):

	Three Months Ended							
	Dec. 31, 2018	Sept. 30, 2018	June 30, 2018	March 31, 2018	Dec. 31, 2017	Sept. 30, 2017	June 30, 2017	March 31, 2017
Statements of Operations Data:								
Revenue	$ —	$ —	$ —	$ —	$ —	$ —	$ —	$ —
Loss from operations	(17,930)	(16,702)	(14,025)	(11,306)	(8,170)	(7,015)	(15,731)	(4,811)
Net loss	(16,679)	(16,015)	(13,660)	(11,161)	(8,036)	(6,913)	(15,731)	(4,811)
Net loss attributable to common stockholders	(16,679)	(16,015)	(13,748)	(11,161)	(8,036)	(6,919)	(15,387)	(5,165)
Net loss per share attributable to common stockholders, basic and diluted	(0.50)	(0.49)	(3.13)	(4.53)	(3.59)	(3.44)	(8.90)	(3.59)

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

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our Principal Executive Officer (our Chief Executive Officer) and Principal Financial Officer (our Chief Operating and Financial Officer), has evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2018. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2018, our Principal Executive Officer and Principal Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Internal Control Over Financial Reporting

Management's Report on Internal Control Over Financial Reporting

This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the company's independent registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended December 31, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item 10 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders and is incorporated herein by reference.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders and is incorporated herein by reference.

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

The information required by this Item 12 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders and is incorporated herein by reference.

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

The information required by this Item 13 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item 14 will be included in our definitive proxy statement to be filed with the SEC with respect to our 2019 Annual Meeting of Stockholders and is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

1. Financial Statements

For a list of the financial statements included herein, see Index to the Consolidated Financial Statements on page 144 of this Annual Report on Form 10-K, incorporated into this Item by reference.

2. Financial Statement Schedules

Financial statement schedules have been omitted because they are either not required or not applicable or the information is included in the consolidated financial statements or the notes thereto.

3. Exhibits

The exhibits required by Item 601 of Regulation S-K and Item 15(b) of this Annual Report on Form 10-K are listed in the Exhibit Index immediately preceding the signature page of this Annual Report on Form 10-K. The exhibits listed in the Exhibit Index are incorporated by reference herein.

ITEM 16. FORM 10-K SUMMARY

None.

Exhibit Index

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant (Incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-38541) filed with the Securities and Exchange Commission on June 25, 2018).
3.2	Amended and Restated By-laws of the Registrant (Incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-38541) filed with the Securities and Exchange Commission on June 25, 2018).
4.1	Specimen Common Stock Certificate (Incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1/A (File No. 333-225178) filed with the Securities and Exchange Commission on June 8, 2018).
4.2	Second Amended and Restated Investors' Rights Agreement by and among the Registrant and certain of its stockholders dated April 2, 2018 (Incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.1#	2016 Stock Option and Grant Plan, as amended, and forms of award agreements thereunder (Incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.2#	2018 Stock Option and Incentive Plan and forms of award agreements thereunder (Incorporated by reference to Exhibit 10.2 to the Registrant's Registration Statement on Form S-1/A (File No. 333-225178) filed with the Securities and Exchange Commission on June 8, 2018).
10.3#	Senior Executive Cash Incentive Bonus Plan (Incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.4#	Form of Employment Agreement (Incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.5#	Employment Agreement by and between the Registrant and Jason Ryan, effective January 1, 2019 (Incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K (File No. 001-38541) filed with the Securities and Exchange Commission on November 19, 2018).
10.6#	Form of Director Indemnification Agreement (Incorporated by reference to Exhibit 10.5 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.7	License Agreement by and between the Registrant and President and Fellows of Harvard College, dated as of November 2, 2016 (Incorporated by reference to Exhibit 10.6 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.8	License Agreement by and between the Registrant and Novartis International Pharmaceutical Ltd., dated as of April 3, 2017 (Incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.9	Collaboration Agreement by and between the Registrant and Be The Match BioTherapies, dated as of November 10, 2017 (Incorporated by reference to Exhibit 10.8 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).

Exhibit Number	Description
10.9.1*†	Project Rider #2, effective as of June 26, 2018, pursuant to the Collaboration Agreement by and between the Registrant and Be The Match BioTherapies, dated as of November 10, 2017.
10.9.2*†	Project Rider #3, effective as of September 6, 2018, pursuant to the Collaboration Agreement by and between the Registrant and Be The Match BioTherapies, dated as of November 10, 2017.
10.9.3*	Amendment #1 to Project Rider #1, effective as of December 6, 2018, pursuant to the Collaboration Agreement by and between the Registrant and Be The Match BioTherapies, dated as of November 10, 2017.
10.10	Clinical Trial Agreement by and between the Registrant and Regents of the University of Minnesota, dated as of January 22, 2018 (Incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.11	Master Development and Manufacturing Agreement by and between the Registrant and Bachem Americas, Inc., dated as of February 13, 2018 (Incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1/A (File No. 333-225178) filed with the Securities and Exchange Commission on June 18, 2018).
10.12	Exclusive Research, Development Option and License Agreement by and between the Registrant and Heidelberg Pharma Research GmbH, dated as of March 1, 2018 (Incorporated by reference to Exhibit 10.11 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.13	Sublease Agreement by and between the Registrant and Surface Oncology, Inc., dated as of September 15, 2016 (Incorporated by reference to Exhibit 10.12 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.13.1	First Amendment to Sublease Agreement, dated as of May 30, 2018, by and between the Registrant and Surface Oncology, Inc. (Incorporated by reference to Exhibit 10.14 to the Registrant's Registration Statement on Form S-1/A (File No. 333-225178) filed with the Securities and Exchange Commission on June 8, 2018).
10.14	Sublease by and between the Registrant and Novartis Institutes for BioMedical Research, Inc., dated as of May 4, 2018 (Incorporated by reference to Exhibit 10.13 to the Registrant's Registration Statement on Form S-1 (File No. 333-225178) filed with the Securities and Exchange Commission on May 24, 2018).
10.15	Agreement, dated as of May 31, 2018, by and between the Registrant and The Richmond Group, Inc. (Incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q (File No. 001-38541) filed with the Securities and Exchange Commission on November 8, 2018).
21.1*	List of Subsidiaries of the Registrant.
23.1*	Consent of KPMG LLP, independent registered public accounting firm.
24.1*	Power of Attorney (included on signature page to this Annual Report on Form 10-K).
31.1*	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.

Exhibit Number	Description
32.1**	Certifications of the Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101INS	XBRL Instance Document.
101SCH	XBRL Taxonomy Extension Schema Document.
101CAL	XBRL Taxonomy Extension Calculation Linkbase Document.
101LAB	XBRL Taxonomy Extension Labels Linkbase Document.
101PRE	XBRL Taxonomy Extension Presentation Linkbase Document.
101DEF	XBRL Taxonomy Extension Definition Linkbase Document.

* Filed herewith.
** Furnished herewith.
# Represents management compensation plan, contract or arrangement.
† Application has been made to the Securities and Exchange Commission for confidential treatment of certain provisions. Omitted material for which confidential treatment has been requested has been filed separately with the Securities and Exchange Commission.

SIGNATURES

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

MAGENTA THERAPEUTICS, INC.

Date: March 19, 2019

By: /s/ Jason Ryan

Jason Ryan
Chief Operating and Financial Officer
(Principal Financial and Accounting Officer)

POWER OF ATTORNEY

Each person whose individual signature appears below hereby authorizes and appoints Jason Gardner and Jason Ryan, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file any and all amendments to this annual report on Form 10-K and to file the same, with all exhibits thereto, and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their or his substitute or substitutes may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Jason Gardner, D.Phil. Jason Gardner, D.Phil.	President, Chief Executive Officer and Director (Principal Executive Officer)	March 19, 2019
/s/ Jason Ryan Jason Ryan	Chief Operating and Financial Officer (Principal Financial and Accounting Officer)	March 19, 2019
/s/ Jeffrey Albers Jeffrey Albers	Director	March 19, 2019
/s/ Michael W. Bonney Michael W. Bonney	Director	March 19, 2019
/s/ Bruce Booth, D.Phil. Bruce Booth, D.Phil.	Director	March 19, 2019
/s/ Alexis A. Borisy Alexis A. Borisy	Director	March 19, 2019
 Blake Byers, Ph.D	Director	March 19, 2019
/s/ Thomas O. Daniel, M.D. Thomas O. Daniel, M.D.	Director	March 19, 2019
/s/ Amy L. Ronneberg Amy L. Ronneberg	Director	March 19, 2019
/s/ David T. Scadden, M.D. David T. Scadden, M.D.	Director	March 19, 2019

Directors

Michael W. Bonney
Chair
Magenta Therapeutics, Inc.

Jeffrey Albers
President and Chief Executive Officer
Blueprint Medicines Corporation

Bruce Booth
Partner
Atlas Venture

Alexis A. Borisy
Partner
Third Rock Ventures

Blake Byers
Partner
GV

Thomas O. Daniel
Chairman
Vividion Therapeutics, Inc.

Jason Gardner
President and Chief Executive Officer
Magenta Therapeutics, Inc.

Amy L. Ronneberg
Chief Financial Officer
Be The Match

David T. Scadden
Gerald and Darlene Jordan Professor of Medicine
Harvard University

Executive Officers

Jason Gardner
President and Chief Executive Officer

Michael P. Cooke
Chief Scientific Officer

John C. Davis, Jr.
Chief Medical Officer

Christina K. Isacson
Chief Business Officer

Jason Ryan
Chief Operating and Financial Officer

Zoran Zdraveski
Chief Legal Officer and Corporate Secretary

Transfer Agent and Registrar
Shareholder correspondence should be mailed to:
Computershare Trust Company, N.A.
P.O. Box 505000
Lousiville, KY 40233

Shareholder website:
www-us.computershare.com/investor

Shareholder online inquiries:
www-us.computershare.com/Investor/Contact/Enquiry

Shareholder Services:
Toll-Free Number:
877-239-3295

Independent Registered Public Accounting Firm
KPMG LLP
One Broadway, 15th Floor
Cambridge, MA 02142
Phone: 617 475 4700

Stockholders' Meeting
Date: Friday, June 7th, 2019 at 9:00 a.m., Eastern Time
Location: Goodwin Procter 100 Northern Avenue Boston, MA 02210

Stock Symbol
NASDAQ: MGTA

Investor Relations
Manisha Pai
Vice President, Investor Relations & Corporate Communications
Email: mpai@magentatx.com

Form 10-K
A copy of the Company's 10-K, filed with the Securities and Exchange Commission, is available without charge upon written request to:
Magenta Therapeutics
Attn: Investor Relations
100 Technology Square, 5th Floor
Cambridge, MA 02139

Corporate Headquarters
Magenta Therapeutics
100 Technology Square, 5th Floor
Cambridge, MA 02139
Email: info@magentatx.com
URL: www.magentatx.com

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