GLYCOMIMETICS INC
Form 10-K
March 06, 2018
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-K

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d)

OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2017

Commission file number 001-36177

GlycoMimetics, Inc.

(Exact name of Registrant as specified in its charter)

Delaware	06-1686563
(State or other jurisdiction of incorporation or organization)	(IRS Employer Identification No.)

9708 Medical Center Drive

Rockville, Maryland	20850
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (240) 243-1201

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

Title of Each Class:	Name of Each Exchange on which Registered
Common Stock, $0.001 par value	The Nasdaq Stock Market

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

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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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained to the best of 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 emerging growth company. See 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	(Do not check if a smaller reporting company)	Smaller reporting company
	Emerging growth company	

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

As of June 30, 2017, the last business day of the registrant's last completed second quarter, the aggregate market value of the Common Stock held by non-affiliates of the registrant was approximately $262.5 million based on the closing price of the registrant's Common Stock, as reported by the Nasdaq Global Market, on such date.

At February 28, 2018, 34,359,799 shares of GlycoMimetics, Inc.'s Common Stock, $0.001 par value per share, were outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of GlycoMimetics, Inc.'s definitive proxy statement, to be filed pursuant to Regulation 14A under the Securities Exchange Act of 1934, for its 2018 Annual Meeting of Stockholders are incorporated by reference in Part III of this Form 10-K.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, or this Annual Report, contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, that involve substantial risks and uncertainties. The forward-looking statements are contained principally in Part I, Item 1. “Business,” Part I, Item 1A. “Risk Factors,” and Part II, Item 7. “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” but are also contained elsewhere in this Annual Report. In some cases, you can identify forward-looking statements by the words “may,” “might,” “will,” “could,” “would,” “should,” “expect,” “intend,” “plan,” “objective,” “anticipate,” “believe,” “estimate,” “predict,” “project,” “potential,” “ongoing,” or the negative of these terms, or other comparable terminology intended to identify statements about the future. These statements 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 the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Annual Report, we caution you that these statements are based on a combination of facts and factors currently known by us and our expectations of the future, about which we cannot be certain. Forward-looking statements include statements about:

- our plans to develop and commercialize our glycomimetic drug candidates;
- our ongoing and planned clinical trials for our drug candidates GMI-1271 and GMI-1359, including the timing of initiation of and enrollment in the trials, the timing of availability of data from the trials and the anticipated results of the trials;
- our ability to achieve anticipated milestones and potential royalties under our collaboration with Pfizer for our drug candidate rivipansel and the timing and results of the ongoing Phase 3 clinical trial of rivipansel;
- the timing of and our ability to obtain and maintain regulatory approvals for our drug candidates;
- the clinical utility of our drug candidates;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position;
- our ability to identify additional drug candidates with significant commercial potential that are consistent with our commercial objectives;
- our estimates regarding future revenues, expenses and needs for additional financing; and
- our beliefs about our capital expenditure requirements and that our capital resources will be sufficient to meet our anticipated cash requirements through the end of 2019.

You should refer to Item 1A. “Risk Factors” section of this Annual Report for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this Annual Report will prove to be accurate. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. We undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Annual Report.

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PART I

ITEM 1.BUSINESS

Overview

We are a clinical stage biotechnology company focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Using our expertise in carbohydrate chemistry and knowledge of carbohydrate biology, we are developing a pipeline of proprietary glycomimetics designed to inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection. We believe this represents an innovative approach to drug discovery to treat a wide range of diseases.

We are focusing our initial efforts on drug candidates for rare diseases that we believe will qualify for orphan drug designation. Our first drug candidate, rivipansel, is being developed for the treatment of vaso-occlusive crisis, or VOC, a debilitating and painful condition that occurs periodically throughout the life of a person with sickle cell disease, or SCD. We have entered into a collaboration with Pfizer Inc., or Pfizer, for the further development and potential commercialization of rivipansel worldwide. Rivipansel has received fast track designation from the U.S. Food and Drug Administration, or FDA, as well as orphan drug designation from the FDA in the United States and from the European Medicines Agency, or EMA, in the European Union, or EU. We believe the clinical progress of rivipansel provides evidence of the significant potential of our lead program and our proprietary glycomimetics platform. Building on our experience with rivipansel, we are developing our second most advanced drug candidate, GMI-1271, to be used in combination with chemotherapy to treat either acute myeloid leukemia, or AML, or multiple myeloma, or MM, both of which are life-threatening hematologic cancers, and potentially other hematologic cancers as well. We are also developing a third drug candidate, GMI-1359, which is being evaluated in an ongoing Phase 1 clinical trial in healthy volunteers.

Our proprietary glycomimetics platform is based on our expertise in carbohydrate chemistry and our understanding of the role carbohydrates play in key biological processes. Most human proteins are modified by the addition of complex carbohydrates to the surface of the proteins. The addition of these carbohydrate structures affects the functions of these proteins and their interactions with other molecules. Our initial research and development efforts have focused on drug candidates targeting selectins, which are proteins that serve as adhesion molecules and bind to carbohydrates that are involved in the inflammatory component and progression of a wide range of diseases, including hematologic disorders, cancer and cardiovascular disease. For example, we believe that members of the selectin family play a key role in the onset and progression of VOC and also in tumor metastasis and resistance to chemotherapy. Inhibiting specific carbohydrates from binding to selectins has long been viewed as a potentially attractive approach for therapeutic intervention. The ability to successfully develop drug-like compounds that inhibit binding with selectins, known as selectin antagonists, has been limited by the complexities of carbohydrate chemistry. We believe our expertise in carbohydrate chemistry and our understanding of carbohydrate-protein binding interactions enable us to design selectin antagonists and other glycomimetics that may inhibit the disease-related functions of certain carbohydrates in order to develop novel drug candidates to address unmet medical needs.

Rivipansel is a glycomimetic drug candidate that acts as a pan-selectin antagonist, meaning it binds to all three members of the selectin family, E-, P- and L-selectin. We believe that rivipansel, by acting as a pan-selectin antagonist, inhibits the role that selectins play in VOC for people with SCD. VOC, one of the most severe complications of SCD, can result in acute ischemic tissue injury at one or more sites, with inflammation and pain of varying degrees of severity. The standard of care in the United States for people experiencing VOC is to manage its symptoms, which typically includes hospitalization, narcotic pain management and hydration. We believe that

rivipansel, if approved, would be the first drug to interrupt the underlying cause of VOC, thereby potentially reducing the use of narcotics for pain management and enabling patients to leave the hospital more quickly.

We have completed four clinical trials of rivipansel involving a total of 163 subjects. In April 2013, we completed a Phase 2 clinical trial in which 76 patients hospitalized for VOC, ranging from 12 to 60 years old, were treated with the standard of care plus either rivipansel or placebo. In this trial, patients treated with rivipansel experienced reductions in the time to reach resolution of VOC, length of hospital stay and use of opioid analgesics for pain management, in each

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case as compared to patients receiving placebo. This improvement was seen in both adult and pediatric patients. Adverse event rates and severity were comparable between those treated with rivipansel and those receiving placebo.

Since the completion of our Phase 2 clinical trial of rivipansel in 2013, Pfizer has been responsible for the further clinical development, regulatory approval and potential commercialization of rivipansel. Pfizer enrolled the first patient in a Phase 3 clinical trial in June 2015 and has announced that it expects to complete enrollment in this trial in the second half of 2018, with preliminary results expected to be announced by the end of 2018. Under our license agreement with Pfizer, we are eligible to receive payments of up to $115.0 million upon the achievement of specified development milestones, up to $70.0 million upon the achievement of specified regulatory milestones, and up to $135.0 million upon the achievement of specified levels of annual net sales of licensed products. We are also eligible to receive tiered royalties, with percentages ranging from the low double digits to the low teens, based on net sales of rivipansel worldwide, subject to reductions in specified circumstances. Under a separate research agreement with the University of Basel, or the University, we have agreed to pay 10% of any future milestone payments and royalties we may receive from Pfizer with respect to rivipansel.

We are developing a pipeline of other drug candidates based on our expertise in carbohydrate chemistry, including compounds that are designed to be specific to particular selectins. We are developing GMI-1271, a specific E-selectin inhibitor, to be used in combination with chemotherapy to treat patients with AML, MM and potentially other hematologic cancers.

E-selectin plays a critical role in binding cancer cells within vascular niches in the bone marrow, which prevents the cells from entering circulation where they can be more readily killed by chemotherapy. In separate animal studies, GMI-1271 mobilized AML and MM cancer cells out of the bone marrow, making them more sensitive to chemotherapy. In both the AML and MM studies, tumor burden was significantly reduced in the animals treated with a combination of chemotherapy and GMI-1271 as compared to animals treated with chemotherapy alone. In addition, the combination of GMI-1271 with chemotherapy resulted in improved survival rates for the treated animals, compared to chemotherapy alone. In other animal studies, GMI-1271 appeared to also protect normal cells from some of the side effects of chemotherapy. Common side effects of chemotherapy include bone marrow toxicity resulting in neutropenia, which is an abnormally low number of neutrophils, the white blood cells that serve as the primary defense against infection, and mucositis, which is the inflammation and sloughing of the mucous membranes lining the digestive tract. Animals treated with GMI-1271 and chemotherapy had less severe neutropenia and mucositis and lower bone marrow toxicity as compared to animals treated with chemotherapy alone. We believe that treatment with GMI-1271 results in lower bone marrow toxicity due to its inhibition of E-selectin, which inhibition makes stem cells in the bone marrow divide less frequently, thereby protecting them from chemotherapy agents that target rapidly dividing cells.

We have completed an initial Phase 1 trial in healthy volunteers for GMI-1271 and in May 2017 we completed enrollment in a Phase 1/2 clinical trial in defined populations of patients with AML. In December 2017, at the annual meeting of the American Society of Hematology, or ASH, we presented clinical data that showed high remission rates, improved overall survival and improved duration of survival, all compared to historical controls, which have been derived from results from third party clinical trials evaluating standard chemotherapy. In addition, the data suggested a favorable safety, pharmacokinetic, or PK, and biomarker profile for GMI-1271. We have also initiated a Phase 1 multiple ascending dose-escalation trial of GMI-1271 in defined populations of patients with MM and plan to continue enrollment of the trial in 2018. We anticipate initial topline data in the first quarter of 2019 in this trial.

We are developing an additional drug candidate, GMI-1359, that simultaneously targets both E-selectin and a chemokine receptor known as CXCR4. Since E-selectin and CXCR4 are both adhesion molecules that keep cancer

cells in the bone marrow, we believe that targeting both E-selectin and CXCR4 with a single compound could improve efficacy in the treatment of cancers that affect the bone marrow such as AML and MM, as compared to targeting CXCR4 alone. GMI-1359 is currently being evaluated in a Phase 1 single-dose escalation trial in healthy volunteers. In this trial, volunteer participants receive a single injection of GMI-1359, after which they are evaluated for safety, tolerability, PK and pharmacodynamics. The randomized, double-blind, placebo-controlled, escalating dose study is being conducted at a single site in the United States.

In addition to our programs described above, we are also advancing other preclinical-stage programs. These programs include small-molecule glycomimetic compounds that inhibit galectin-3, which we believe may have potential to be used for the treatment of fibrosis, cancer and cardiovascular disease.

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We have retained the worldwide development and commercialization rights to all of our drug candidates other than rivipansel.

Our intellectual property portfolio includes ownership of, or exclusive rights to, issued patents and pending patent applications claiming fundamental features of glycomimetic therapeutics, as well as those claiming methods of use for and chemical modifications of our drug candidates. Given the importance of our intellectual property portfolio to our business operations, we intend to vigorously enforce our rights and defend against challenges that have arisen or may arise in this area. Our issued patents directed to rivipansel and methods of use are expected to expire between 2023 and 2030. We also have issued patents which cover GMI-1271 and methods of use that expected to expire between 2032 and 2033. In addition, we have several pending patent applications covering GMI-1271 and/or methods of using it, the last expiring of which, if issued, currently would be predicted to expire in 2037.

"GlycoMimetics," the GlycoMimetics logo and other trademarks or service marks of GlycoMimetics, Inc. appearing in this Annual Report are the property of GlycoMimetics, Inc. This Annual Report contains additional trade names, trademarks and service marks of others, which are the property of their respective owners.

Our Strategy

Our goal is to be the leader in the discovery, development and commercialization of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Leveraging the potentially broad applicability of our proprietary glycomimetics platform, our initial focus is to internally develop and advance orphan drug candidates targeted at hematologic cancers and other diseases, and to out-license any drug candidates we may develop that are targeted at larger market opportunities. The key elements of our strategy are to:

- Advance and complete the clinical development of GMI-1271 for the treatment of AML. We are building on our experience developing rivipansel to investigate GMI-1271 for the treatment of AML as an adjunct to standard chemotherapy. We have completed enrollment in a Phase 1/2 dose-escalation clinical trial in defined populations of patients with AML. In May 2017, GMI-1271 received breakthrough therapy designation from the FDA for the treatment of adult patients with relapsed or refractory AML and also received orphan designation from the European Commission for the treatment of AML. We anticipate initiating a pivotal Phase 3 clinical trial in mid-2018 for GMI-1271 in patients with relapsed or refractory AML. We have retained worldwide development and commercialization rights to GMI-1271.
- Advance the clinical development of GMI-1271 for the treatment of MM. We are enrolling a Phase 1 multiple dose-escalation clinical trial for patients with MM who have not responded optimally to standard chemotherapy. We are currently enrolling patients at multiple clinical trial sites in Europe.
- Advance the clinical development of GMI-1359 for the treatment of cancer. We are developing GMI-1359, which simultaneously inhibits both E-selectin and CXCR4, for potential use in the treatment of cancers with significant bone marrow involvement, such as hematologic cancers including AML and MM and certain solid tumors such as breast and prostate cancer. We are currently conducting a Phase 1 single-dose escalation trial in healthy volunteers. We have retained worldwide development and commercialization rights to GMI-1359.
- Support Pfizer's further development of rivipansel. We will continue to support Pfizer, if requested, as Pfizer proceeds with further clinical development of rivipansel, including the Phase 3 clinical trial, and pursues regulatory approval of rivipansel. We expect to use any milestone and royalty payments that we may receive from Pfizer to accelerate the development of our other drug candidates.

Identify and develop additional novel selectin antagonists to address unmet medical needs with significant market potential. We believe our glycomimetics platform will enable us to develop a broad pipeline of potential drug candidates that may be orphan drugs or may address larger market opportunities. We have identified a highly potent E-selectin antagonist which is being explored for subcutaneous delivery and which we believe could be of value in potential major market opportunities, such as the treatment of certain cancers and cardiovascular disease.

- Apply our insights and our glycomimetics platform to other carbohydrate targets beyond selectins. We have identified additional opportunities where carbohydrates play critical roles in disease processes and

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where we believe we can apply our platform to create targeted glycomimetic drugs. We have designed inhibitors that specifically block the binding of galectin-3 to carbohydrate structures. Galectin-3 is a protein that is known to play critical roles in many pathological processes, including fibrosis, inflammation, cancer and cardiovascular disease. We plan to optimize these compounds and conduct preclinical experiments in in 2018 to further characterize the effects of galectin-3 inhibitors on immune processes and anti-fibrotic activity. We are also designing other galectin inhibitors that we believe could be used to treat various diseases.

Our Platform

Our proprietary glycomimetics platform is based on our expertise in carbohydrate chemistry and our understanding of the role carbohydrates play in key biological processes. Carbohydrate structures on cell surfaces are responsible for complex carbohydrate-protein binding interactions. Inhibiting these binding interactions affects the functions of these proteins and their interactions with other molecules. We believe our expertise enables us to design specific glycomimetic molecules that can mimic carbohydrate structures and thereby inhibit their disease-related functions.

Our initial focus is on selectin antagonists, which we believe have the potential to address unmet medical needs in a number of orphan and large market opportunities. Selectins have been shown to play a key role in a wide range of diseases, including hematologic disorders, cancer and cardiovascular disease.

Our initial drug design efforts are focused on a naturally occurring, three-dimensional complex carbohydrate core structure known as the Lewis structure. This core structure is naturally modified in a variety of ways to form many different functional carbohydrates. These variations determine the biological functions of the carbohydrates, including functions related to conditions such as inflammatory diseases, cancer and infection. Accordingly, we believe that this structure provides the foundation for the design of glycomimetic drug candidates that could be used to address a variety of diseases.

Once we identify a carbohydrate structure involved in a disease pathway, we design molecules that mimic that carbohydrate structure and inhibit its disease-related functions by binding to the carbohydrate's target receptor, thereby blocking the binding by the native carbohydrate itself. For example, one of the naturally modified Lewis structures binds to selectins, which play a key role in VOC. Rivipansel mimics that carbohydrate structure and accordingly binds to selectins, which we believe thereby inhibits the progression of VOC. In addition, our glycomimetic molecules are designed to have greater affinity to the carbohydrate's target receptor than does the native carbohydrate. This means that the glycomimetic molecules possess stronger intermolecular forces between themselves and the target receptors, and thus "outcompete" the native carbohydrates in binding to the relevant target receptors, thereby inhibiting their disease-related functions. Using our glycomimetics platform, we have designed and synthesized a proprietary library of these structures targeting different biological processes.

Our glycomimetics platform includes intellectual property, know-how, expertise, proprietary biological information and biochemical assays, all of which support the rational design of potent glycomimetic compounds. These include:

- Know-how to successfully mimic the Lewis structure, which is common to a number of functional carbohydrates.
- Use of empirical methods to determine critical interactions between variations of a particular functional carbohydrate and its target molecule.
- Application of the empirically determined bioactive structure of the functional carbohydrate for docking into the binding area of the crystal structure of the target molecule.
- Expertise in stabilizing the bioactive core of glycomimetic compounds and increasing the number of interaction contact points to improve affinity.
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Experience and technology in synthetic organic chemistry required for the specialized synthesis of carbohydrates and their modifications.

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· Proprietary assays to determine the binding characteristics, inhibitory activity and biological activity of glycomimetic compounds.

Our Pipeline

We have discovered our drug candidates internally through a rational drug design approach that couples our expertise in carbohydrate chemistry with our knowledge of carbohydrate biology. We are actively developing glycomimetic drug candidates based on this expertise. Our drug candidates and their target indications and development status are summarized in the chart below.

Rivipansel —Targeting Selectins to Treat VOC

Rivipansel is being developed to treat VOC with the goal of reducing duration of VOC episodes, length of hospital stay and use of opioid analgesics for pain management. In our Phase 2 clinical trial, patients treated with rivipansel plus the standard of care demonstrated improvement in these endpoints, in each case as compared to patients receiving placebo plus the standard of care.

Sickle Cell Disease and VOC

SCD is a genetic disease that, according to the Centers for Disease Control and Prevention, or CDC, affects millions of people throughout the world, including an estimated 100,000 people in the United States and an estimated 60,000 people in Europe. Patients with SCD have chronic and acute damage to their tissue and organs. One of the most common and severe complications of SCD is vaso-occlusive crisis, or VOC, which is the occurrence of unpredictable episodes of acute pain, often very excruciating, in the affected parts of the body. Recurrent episodes may cause irreversible organ damage. The CDC estimates that VOC resulted in approximately 75,000 hospitalizations in the United States in 2010. According to the National Hospital Discharge Survey conducted by the National Center for Health Statistics, these hospitalizations have an average duration of approximately six days.

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Among both adults and children with SCD, VOC is the most common reason for seeking medical attention resulting in hospitalization. As there are no approved therapies that interrupt VOC once it has started or that treat the underlying ischemic event, the standard of care for people experiencing VOC is limited to supportive care consisting of pain management, hydration and treatment of any precipitating events such as infection and inflammatory conditions.

Market Opportunity for Rivipansel in SCD

We believe that effective, on-demand (as needed) treatment for VOC could provide significant clinical and pharmacoeconomic benefit. According to the U.S. Agency for Healthcare Research and Quality, the average hospital charges in the United States for a patient treated for VOC were approximately $20,000 in 2006. In some states, these charges may be substantially higher. For example, according to the California Office of Statewide Health Planning and Development, the average hospital charges for a patient treated for VOC in California were over $40,000 in 2006. A reduction in the length of a hospital stay following treatment with rivipansel could significantly reduce these costs of care.

Additionally, if rivipansel is shown to be safe and effective in reducing the duration of VOC in hospitalized patients, it could also be tested to determine if hospitalization could be prevented with use of rivipansel in the emergency department, or if VOC could be managed safely and effectively in the home or in an outpatient setting through a self-administered dosage form, thereby avoiding costly emergency department visits. We believe that uses in each of these settings represent potentially significant market opportunities.

The Role of Selectins in VOC

The cause of vascular occlusion involves both an inflammatory component and a mechanical component. In the inflammatory component, white blood cells begin to roll along and then adhere to the endothelium, the thin layer of cells that lines the interior surface of blood vessels. These white blood cells then become activated and express adhesion receptors known as integrins, which bind and form aggregates with platelets, red blood cells and other white blood cells. These cell aggregates are responsible for the mechanical component of vascular occlusion, in which rigid sickled red blood cells are more easily caught in the post-capillary venules, which are very small blood vessels connecting the capillaries and the veins. The resulting vascular occlusion causes slowing of blood flow in the post-capillary venules, contributing to inadequate oxygen supply in the local tissue, known as ischemia, which in turn causes further tissue inflammation and pain.

The development of VOC is illustrated in the following diagram:

Selectins are important in this process because they act as adhesion molecules and play a key role in the initial recognition and binding of white blood cells to the endothelial cells, and their formation of aggregates with platelets, red blood cells and other white blood cells. White blood cells express carbohydrates on their surfaces that bind to E-selectin that is present on inflamed vascular endothelium. White blood cells bound to E-selectin on the endothelial cells then become activated and act as adhesion sites for platelets, red blood cells and other white blood cells, thereby leading to the formation of an occlusion. Rivipansel is a glycomimetic drug candidate designed to inhibit binding of all three types of selectins and inhibit the selectin-mediated recognition and binding of white blood cells to the endothelium. The

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rationale for the development of rivipansel to treat VOC is that, by blocking these steps in the vaso-occlusive process, it has the potential to decrease the duration and intensity of VOC.

Limitations of the Current Standard of Care for VOC

Although bone marrow transplant is currently available and can be curative for SCD, its use is greatly limited by the lack of availability of matched donors and by the risk of serious complications, including graft versus host disease and infection and death.

Until 2017, the only drug approved to treat SCD was hydroxyurea. Hydroxyurea, available in both generic and branded formulations, is a once daily oral treatment intended to reduce the frequency of VOC in patients with SCD who have recurrent VOC episodes. While hydroxyurea has been shown to reduce the frequency of hospitalization due to VOC, in some patient groups, it is not effective in relieving symptoms or accelerating the resolution of an ongoing VOC episode. Moreover, hydroxyurea is not suitable for all patients. Its uptake and effectiveness can be limited by a lack of compliance to the dosing regimen, inconsistent patient responses, variable tolerability and concerns regarding long-term toxicity [and other adverse side effects]. In particular, hydroxyurea is labeled to inform patients that it can cause a severe decrease in the number of blood cells in a patient's bone marrow, which may increase risks that the patient will develop a serious infection or bleeding, and that it may increase the risk that the patient will develop certain cancers. Furthermore, hydroxyurea is not effective in relieving symptoms or accelerating the resolution of an ongoing VOC episode.

In July 2017, the FDA approved Endari, a twice-daily, oral, prophylactic therapy intended to reduce the acute complications of SCD in adult and pediatric patients 5 years of age and older. Common side effects of Endari include constipation, nausea, headache, abdominal pain, cough, pain in the extremities, back pain and chest pain. Given its recent launch in December 2017, real world experience with Endari is limited.

Since available therapies do not address the underlying pathophysiology of an ongoing VOC episode, supportive care with opioid narcotics and hydration remain the current standard of care for VOC until the event runs its natural course. Pain management often starts with oral medications taken at home at the onset of pain. However, if the pain is not relieved, or if it progresses, patients typically seek medical attention in a clinic setting or emergency department. Pain that is not controlled in these settings typically requires hospitalization for more potent pain medications, typically administered intravenously. The patient must stay in the hospital to receive these intravenous pain medications and fluids until the VOC resolves and the pain subsides. Use of narcotics can lead to tissue or organ damage and resulting complications and morbidities, prolonged hospital stays and associated continuation of pain and suffering. Treatment of pain with IV narcotics and management of VOC-related complications typically require hospital stays ranging from a few days to a few weeks, with an average length of stay of approximately six days. Other supportive measures during hospitalization include supplemental oxygen and treatment of any concurrent infections or other conditions.

In light of the debilitating effects of VOC and the associated high costs of care, there is a significant unmet medical need for treatment that can be taken at the onset of a VOC episode that selectively targets the underlying inflammatory ischemic event in order to reduce the severity and duration of VOC in SCD patients. We believe that rivipansel can potentially satisfy this unmet medical need.

Rivipansel Clinical Results

We completed a Phase 2 clinical trial of rivipansel in sickle cell patients hospitalized for VOC. This trial was a randomized, double-blind, placebo-controlled trial at 22 sites in the United States and Canada evaluating the safety, efficacy and PK of multiple IV doses of rivipansel or placebo in 76 patients hospitalized for VOC, ranging from 12 to 60 years old. Of these patients, 43 received rivipansel and 33 received placebo, in both cases in addition to the standard of care. Patients receiving rivipansel in the trial received one of two dose levels. Patients in the low dose group received a loading dose of 20 mg/kg, followed by a 10 mg/kg dose every 12 hours. Patients in the high dose group received a loading dose of 40 mg/kg, followed by a 20 mg/kg dose every 12 hours.

In patients receiving rivipansel in this trial, there were reductions in multiple measures related to a VOC episode as compared to patients receiving placebo. Two widely used statistical methods, known as ANCOVA and Kaplan-Meier, were used to analyze the results of this trial. The time to reach resolution of VOC, the primary endpoint of the trial, was

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reduced in the patients receiving rivipansel by a mean of 41.0 hours, as measured by ANCOVA, with a p-value of 0.192, and reduced by a median of 63.3 hours, as measured by Kaplan-Meier, with a p-value of 0.187. P-value is a conventional statistical method for measuring the statistical significance of clinical results. A p-value of 0.05 or less represents statistical significance, meaning that there is a less than 1-in-20 likelihood that the observed results occurred by chance. In addition, in the patients receiving rivipansel, the time to hospital discharge was reduced by a mean of 54.7 hours, as measured by ANCOVA, with a p-value of 0.096, and a median of 83.9 hours, as measured by Kaplan-Meier, with a p-value of 0.092. The time to transition off IV analgesics was reduced by a mean of 47.0 hours, as measured by ANCOVA, with a p-value of 0.137, and a median of 75.7 hours, with a p-value of 0.089, as measured by Kaplan-Meier. The cumulative amount of opioid analgesic administered during hospitalization was reduced by 83%, as measured by ANCOVA, with a p- value of 0.01. Although the Phase 2 clinical trial was not large enough to detect statistically significant differences in these endpoints, other than with respect to the reduction in cumulative amount of opioid analgesic administered, we believe the observed reductions in these measures in patients treated with rivipansel, and the consistency of a positive response across multiple measures, demonstrate the potential benefit of rivipansel.

We believe the favorable effects we observed in our Phase 2 clinical trial are the result of mechanism-based resolution of VOC. Specifically, we believe that by inhibiting selectin-mediated adhesion of white blood cells to the endothelium, rivipansel prevents propagation of VOC and promotes early resolution. Results from the Phase 2 clinical trial provide the first clinical evidence of a positive effect of rivipansel in adult and pediatric patients experiencing VOC. No currently available therapies provide similar benefits to patients in VOC. Based on the data from our Phase 2 clinical trial for rivipansel, we believe rivipansel has the potential to become the first drug approved to treat VOC in both adult and pediatric patient populations.

If rivipansel is demonstrated to be safe and effective for the treatment of VOC, we believe it may show substantial clinical and pharmacoeconomic benefit. If patients treated with rivipansel are discharged more quickly from the hospital, there is potential to reduce the costs of hospitalization, in addition to showing clinical benefit by reduced duration of VOC episodes and reduced use of opioid analgesics for pain management. In addition, if rivipansel is shown to be safe and effective for treating VOC in hospitalized patients, it is possible that it could be tested in patients experiencing VOC who are not hospitalized to determine if hospitalization could be prevented or if pain from VOC could be managed safely and effectively in the home or in an outpatient setting. We believe that uses in each of these settings could represent significant market opportunities for rivipansel. Following the completion of the Phase 2 clinical trial, Pfizer is now responsible for the further clinical development, regulatory approval and commercialization of rivipansel.

Following transfer of the IND, Pfizer has undertaken significant activity to work toward a New Drug Application, or NDA, for rivipansel including, an approved special protocol assessment, or SPA, agreement for the design, endpoints and statistical analysis approach of the Phase 3 clinical trial. Pfizer enrolled the first patient in this Phase 3 clinical trial in June 2015. The Phase 3 trial, entitled “RESET” (Rivipansel: Evaluating Safety, Efficacy and Time to Discharge), is assessing the efficacy and safety of rivipansel for the treatment of VOC in patients hospitalized with sickle cell disease. Pfizer intends to enroll at least 350 subjects with sickle cell disease, aged six and older who are hospitalized for VOC, in this Phase 3, multicenter, randomized, double-blind, placebo-controlled, parallel-group trial in order to evaluate the efficacy and safety of treatment with rivipansel. Trial participants must be receiving treatment with intravenous, or IV, opioids for their VOC and must be able to receive the first dose of rivipansel within 24 hours of initiation of intravenous opioid therapy. The primary endpoint for the trial will be time to readiness-for-discharge. Key secondary endpoints will include time to discharge, cumulative IV opioid consumption and time to discontinuation of IV opioids. Pfizer has announced completion of enrollment in the Phase 3 clinical trial is expected in the second half of 2018.

GMI-1271—Targeting the Bone Marrow Microenvironment to Treat Hematologic Cancers

We are developing GMI-1271, a specific E-selectin antagonist, to be used adjunctively with standard chemotherapy to treat AML, MM and other hematologic cancers. We believe that GMI-1271 may be used as first-line treatment for elderly patients with AML or MM, for patients with relapsed or refractory AML, as well as for patients with relapsed or refractory MM. GMI-1271 targets interactions between cancer cells and the bone marrow microenvironment. In preclinical studies, combining GMI-1271 with chemotherapy made cancer cells more sensitive to chemotherapy. In other preclinical studies, GMI-1271 also reduced some of the toxic effects of chemotherapy, including neutropenia and mucositis, on normal cells.

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GMI-1271 received orphan drug designation from the FDA in May 2015 for the treatment of AML. In June 2016, GMI-1271 received fast track designation from the FDA for the treatment of adult patients with relapsed or refractory AML and elderly patients aged 60 years or older with AML. In May 2017, GMI-1271 received breakthrough therapy designation from the FDA for the treatment of adult patients with relapsed or refractory AML. In May 2017, the European Commission, based on a favorable recommendation from the EMA Committee for Orphan Medicinal Products, granted orphan designation for GMI-1271 for the treatment of AML.

Acute Myeloid Leukemia

AML, a hematologic cancer that is characterized by the rapid growth of abnormal white blood cells that accumulate in the bone marrow and interfere with the production of normal blood cells, is a relatively rare disease, but one that accounts for the largest number of annual deaths from leukemia in the United States. According to the Surveillance, Epidemiology, and End Results Program managed by the National Cancer Institute, there were an estimated 21,380 new cases of AML diagnosed in 2017 in the United States. Approximately 352,000 patients in the world are diagnosed with AML annually. AML caused an estimated 10,590 deaths in 2017 in the United States.

AML is more commonly present in elderly patients, with a median age at diagnosis of 68 years according to the National Cancer Institute. In a review published in the Journal of Clinical Oncology, the median overall survival of patients 60 years old or older was nine months. The overall five-year relative survival rate for all AML patients is 26.9%, and only 3-8% for patients over 60 years old at diagnosis. Relative survival is a statistical measure of net survival that is calculated by comparing observed survival with expected survival from a comparable set of people who do not have AML, in order to measure the excess mortality that is associated with the AML diagnosis.

A number of published studies indicate that only some AML patients who receive chemotherapy achieve a complete response, which is defined as the disappearance of all signs of AML, and that most patients with a complete response will eventually relapse. Patients who do not enter remission are referred to as refractory, meaning that they are resistant to the chemotherapy treatment.

We believe there is a need for new treatment options for elderly patients with AML, as well as those AML patients who relapse or develop refractory disease. Most AML patients with relapsed or refractory disease have no established treatment options and, accordingly, may be referred for participation in clinical studies of potential new therapies. For patients who elect not to participate or are unable to participate, treatment options typically include chemotherapy regimens, hypomethylating agents and supportive care. Further, many elderly patients with AML are too frail to undergo chemotherapy as a result of other medical conditions, and may only be able to tolerate pain comfort or control measures. Without treatment, however, AML is uniformly fatal.

Multiple Myeloma

MM is a hematologic cancer that is characterized by the growth of abnormal white blood cells of the bone marrow that eventually infiltrates various organs and leads to bone destruction, bone marrow failure, including direct and indirect effects on the blood, skeleton, and kidneys. MM is the most frequent tumor that occurs primarily in bone and the second most common hematological malignancy in the United States and Europe. MM accounts for 10% to 15% of hematologic cancers, and 22% of deaths from these cancers. Approximately 114,000 new cases of MM were diagnosed worldwide in 2012. In the United States, according to the National Cancer Institute, an estimated 30,280 people were newly diagnosed with MM in 2017. MM caused an estimated 12,590 deaths in 2017 in the United States. MM is rare in individuals younger than 40 years old and the average age at diagnosis is approximately 70 years. More than 33% of patients are over 75 years of age, making treatment with chemotherapy more complicated due to fewer

treatment options being available, patients being ineligible for transplant, and decreased ability to tolerate sustained chemotherapy due to poor general health.

Despite the fact that recent treatment options for MM have led to improved response rates and increased short-term survival, responses are transient and most patients with MM will ultimately relapse and succumb to their cancer. MM is not considered curable with current approaches. The five-year overall survival rate for all patients in the United States is approximately 50%. Although second and later remissions can be achieved with additional treatment, tumors typically recur more aggressively after each relapse, leading to decreased duration of response and ultimately culminating in the development of treatment-refractory disease. Median survival for treatment-refractory disease typically ranges from five

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months for event-free survival and from nine months for overall survival and responses to treatment are characteristically short, most likely due to resistant disease. This loss of response complicates therapy of patients in later-line treatment, shortens survival and results in high mortality rates.

Role of E-selectin in AML and MM

E-selectin has been shown to play important roles in the progression of AML and MM. This has been observed in several studies, which have shown that levels of E-selectin correlate with tumor infiltration and relapse in AML and survival rates for both diseases. We therefore believe that our E-selectin antagonist, GMI-1271, has the potential to improve the current treatment of patients with AML or MM.

GMI-1271 Preclinical Development

Some leukemia cells, known as blast cells, bind to E-selectin in the bone marrow where they are relatively protected from the effects of chemotherapy. This phenomenon is known as cell adhesion-mediated drug resistance, or CAMDR. We believe that E-selectin inhibition disrupts the adhesion involved in CAMDR and mobilizes blast cells out of the bone marrow and into the bloodstream, making them more susceptible to chemotherapy. We believe that this mechanism of action may allow GMI-1271 to improve chemotherapy response rates, duration of remission and, ultimately, survival in patients with hematologic cancers such as AML.

In one in vivo study in a mouse model of AML, combining GMI-1271 with chemotherapy mobilized AML blast cells and significantly reduced tumor burden as compared to treatment with chemotherapy alone. In an in vitro study, AML cells bound to E-selectin were more resistant to chemotherapy. In a related study, when treated with GMI-1271, the resistance of such cells to chemotherapy was reduced. Tumor cells of patients who have relapsed AML, when tested in the laboratory, bound significantly higher levels of E-selectin than tumor cells of patients at initial diagnosis. Additional preclinical studies in mouse models of AML, in which E-selectin was observed to be upregulated, suggest that AML cells binding to E-selectin have increased chemo-resistance. This is due to the induction of tumor cell survival signaling pathways as a consequence of E-selectin binding. This effect within the bone marrow microenvironment is unique to E-selectin as compared to other vascular adhesion molecules and can be blocked by GMI-1271. The results of this preclinical study were presented in an oral presentation at the 2017 ASH Annual Meeting in December 2017, and we believe the findings provide important information about how treatment with GMI-1271 may improve sensitivity to chemotherapy.

As GMI-1271 disrupts the interactions between cancer cells and bone marrow microenvironment, its mechanism of action is not limited to a single tumor type. In addition to our studies in AML, we have also tested the drug candidate in other cancer models. In three xenograft mouse models of MM, GMI-1271 was evaluated in combination with the proteasome inhibitors, bortezomib or carfilzomib. Treatment of mice with GMI-1271 plus bortezomib or GMI-1271 plus carfilzomib significantly improved survival when compared to chemotherapy alone. In addition, treatment with GMI-1271 resulted in mobilization of MM cells in the peripheral blood past the point of elimination of the drug from plasma and bone marrow, thereby making them more susceptible to chemotherapy. These data provide evidence that GMI-1271 is a potential treatment option to enhance response to chemotherapy in MM. Moreover, in in vivo studies involving animal models of chronic myelogenous leukemia and acute lymphoblastic leukemia, GMI-1271, as an adjunct to standard-of-care chemotherapy, decreased tumor burden and improved survival over standard-of-care

chemotherapy alone.

In addition to its anti-tumor effects, GMI-1271, in animal models, has shown protection against some of the toxicities of chemotherapy. In particular, animals treated with GMI-1271 in combination with chemotherapy had less severe neutropenia and mucositis and lower bone marrow toxicity as compared to animals treated with chemotherapy alone. We believe that treatment with GMI-1271 results in lower bone marrow toxicity due to its inhibition of E-selectin, thereby making hematopoietic stem cells divide less frequently and protecting them from chemotherapy agents that target rapidly dividing cells. Hematopoietic stem cells are blood cells that give rise to all other types of blood cells and are heavily concentrated in the bone marrow. Similar effects have been demonstrated with rivipansel and were published in the journal Nature Medicine in December 2012. Based on these reductions in some of the toxicities of chemotherapy, we are evaluating these effects as secondary efficacy endpoints in our clinical trials.

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GMI-1271 Clinical Trials

In August 2014, we completed a Phase 1 trial of GMI-1271 in healthy volunteers. The single-site Phase 1 trial was a randomized, double-blind, placebo-controlled, single ascending intravenous dose trial. In the trial, we evaluated the safety, tolerability and PK of GMI-1271. Twenty-eight healthy adult subjects were enrolled in cohorts to receive study drug at three dose levels. In the trial, we observed that the subjects tolerated GMI-1271 well, and that the PK for GMI-1271 were as predicted based on preclinical data.

In May 2015, we commenced a multinational, Phase 1/2, open-label trial of GMI-1271 as an adjunct to standard chemotherapy in patients with AML. This trial in males and females with AML was conducted at a number of academic institutions in the United States, Ireland and Australia. The trial consists of two parts. In the Phase 1 portion, escalation testing was performed to determine a recommended GMI-1271 dose in combination with standard chemotherapy to be used in the Phase 2 portion. In the Phase 2 portion of the trial, dose expansion was performed at the recommended dose of 10 mg/kg GMI-1271 in combination with standard chemotherapy. The primary objective of the trial was to evaluate the safety of GMI-1271 in combination with chemotherapy. Secondary objectives were to characterize PK and pharmacodynamics and to observe anti-leukemic activity. A total of 19 patients with relapsed or refractory AML were enrolled and dosed with a single cycle of treatment with GMI-1271 and chemotherapy in the Phase 1 portion of the trial. In the Phase 2 portion, one cohort of 25 patients over 60 years of age with newly diagnosed AML and a second cohort of 47 patients with relapsed or refractory AML were enrolled. Unlike in the Phase 1 portion, some of the patients in the Phase 2 portion were treated with multiple cycles of GMI-1271 with chemotherapy.

In June 2017, we presented interim data from the Phase 2 portion of the trial at the annual meetings of the American Society of Clinical Oncology, or ASCO, and the European Hematology Association, or EHA. In December 2017, we presented further updated data at ASH. In the relapsed or refractory disease arm of the trial, 66 patients had been enrolled. Of the 54 relapsed/refractory patients with AML for whom median follow-up was 6.6 months, the clinical remission (CR+CRi) rate was 43%. The CR/CRi rate is the percentage of patients who achieved remission, with either full or incomplete blood count recovery. The mortality rate among this group at 60 days was 9%. Median overall survival by the Kaplan-Meier method was 9.4 months. This compares favorably to a median overall survival of up to 5.4 months reported for historical, matched controls treated with mitoxantrone, etoposide and cytarabine (MEC) alone. Median duration of remission was 11.1 months for the GMI-1271 treatment group. We believe these results compare favorably to what would be expected in this population, based on published historical results from third party clinical trials evaluating standard chemotherapy in similar patients. Researchers also observed a median E-selectin ligand expression of 35% on bone marrow blasts at baseline, with higher rates among those patients in this cohort who achieved remission. In the newly-diagnosed, treatment-naïve elderly arm of the trial, 25 patients had been enrolled. Among these 25 patients for whom median follow-up with 10.5 months, the clinical remission rate was 68%, with a 75% remission rate for patients with de novo disease and 62% remission rate for patients with secondary AML. Median overall survival by the Kaplan-Meier method was 15.8 months. This compares favorably to a historical median overall survival of approximately 12 months in matched controls treated with 7+3 chemotherapy alone. Median duration of remission was 14.8 months and median event free survival was 11.3 months. Across both populations, GMI-1271 was generally well tolerated with no obvious incremental toxicity observed and lower than expected rates of severe, debilitating, grade 3-4 mucositis reported at 3% incidence reported versus historical rates of 20-25% incidence with MEC induction chemotherapy alone.

In March 2018, we announced our design for a randomized, double-blind, placebo-controlled Phase 3 clinical trial to evaluate GMI-1271 in individuals with relapsed/refractory AML, which design is aligned with guidance received from the FDA. Based on consultations with the FDA, the single pivotal trial is planned to enroll 380 adult patients at approximately 30 to 40 centers in the United States, Canada, Europe and Australia, with enrollment expected to begin in the third quarter of 2018. The primary efficacy endpoint will be overall survival and, importantly, the FDA has indicated that data on overall survival will not need to be censored for transplant in the primary efficacy analysis, meaning that patients who proceed to transplant will continue to be included as part of the survival analysis. The dosing regimen for our planned Phase 3 trial will be the same as for our completed Phase 2 trial. All patients will be treated with standard chemotherapy of either MEC or FAI (fludarabine, cytarabine and idarubicin), with some of the patients randomized to receive GMI-1271 in addition to chemotherapy. Patients receiving GMI-1271 will be dosed for one day prior to initiation of chemotherapy, twice a day through the chemotherapy regimen, and then for two days after the end of chemotherapy. The dose regimen will be fixed, rather than weight-based, which we believe will simplify administration. We plan to offer multiple cycles of consolidation therapy in both arms of the trial for patients who achieve remission. We believe

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that multiple cycles of treatment in patients who respond may drive an even deeper response in patients treated with GMI-1271. If this is the case, it could lengthen the duration of remission with potential for additional benefit on survival.

Key secondary endpoints of the Phase 3 trial will include the incidence of severe mucositis and remission rate, which will be assessed in a hierarchical fashion for potential inclusion in the product labeling, if GMI-1271 is approved for marketing by the FDA. We expect preliminary results from this trial to be available by the end of 2020.

In February 2018, we entered into an agreement with the Haemato Oncology Foundation for Adults in the Netherlands, or HOVON, to initiate clinical trial startup activities to evaluate GMI-1271 in adults with newly diagnosed AML but who cannot tolerate intensive chemotherapy, as well as in patients with myelodysplastic syndrome, or MDS, with a high risk of leukemia. The HOVON trial will be the first to evaluate GMI-1271, together with decitabine, in this underserved population of AML and MDS patients who are not considered by their physicians to be candidates for intensive chemotherapy; these two populations represent a significant potential indication expansion opportunity for GMI-1271. HOVON intends to enroll approximately 140 patients in the clinical trial, including a control arm. Patients will be evaluated after three cycles of therapy, and key efficacy endpoints will include remission rate, disease-free survival and overall survival. The trial is expected to start this year and will be conducted in five countries across Europe.

In December 2015, at the ASH annual meeting, we presented preclinical data suggesting that GMI-1271 could reverse resistance of certain chemotherapies seen in MM. In September 2016, we dosed the first patient in a Phase 1 multiple dose-escalation clinical trial in defined populations of patients with MM who have not responded optimally to standard chemotherapy. In this trial, we are evaluating the efficacy, safety and PK of GMI-1271, combined with bortezomib- or carfilzomib-based chemotherapy, for the treatment of MM. We are currently enrolling patients at multiple clinical trial sites in Europe and anticipate initial topline data in the first quarter of 2019 in this trial.

GMI-1359 - Drug Candidate Targeting E-selectin and CXCR4

The chemokine CXCR4 has emerged as an important pro-inflammatory cytokine that is involved in cell migration throughout the body. Like E-selectin, tumor cells may also use the CXCR4 cellular pathway, contributing to chemoresistance, metastatic disease and ultimately decreased survival. We have an additional drug candidate, GMI-1359, that simultaneously targets both E-selectin and CXCR4. Since E-selectin and CXCR4 are both adhesion molecules that keep cancer cells in the bone marrow, we believe that targeting both E-selectin and CXCR4 with a single compound could improve efficacy in the treatment of cancers that affect the bone marrow, such as hematologic cancers, including AML and MM, and certain solid tumors, such as breast and prostate cancer, as compared to targeting CXCR4 alone. At the ASH annual meeting in December 2016, we presented preclinical data suggesting that GMI-1359 has a unique tumor cell mobilization profile and enhanced the ability of chemotherapy to target and improve survival from a high-risk form of mutated AML. In November 2016, at the annual meeting of the Society for Immunotherapy of Cancer, we presented data from a preclinical study in which GMI-1359, in combination with an antibody against the cancer regulatory programmed death receptor ligand, or PD-L1, shortened time to complete tumor regressions in an animal model of colon cancer. In the preclinical study, the combination therapy also selectively reduced regulatory T cells, which are a class of lymphocytes that suppress immune responses, in the tumor, and created a more favorable immune-mediated anti-tumor environment.

We are currently conducting a first-in-human Phase 1 single-dose escalation trial of GMI-1359 in healthy volunteers. In this trial, volunteer participants receive a single injection of GMI-1359, after which the subjects are evaluated for safety, tolerability, pharmacokinetics and pharmacodynamics. The randomized, double-blind placebo controlled escalating dose study is being conducted at a single site in the United States.

Galectin Inhibitors

Using our glycomimetics platform, we have designed galectin-3 inhibitors that specifically block the binding of galectin-3 to carbohydrate structures. Galectin-3 is a protein that is known to play critical roles in many pathological processes, including fibrosis, checkpoints in T-cell exhaustion during cancer immunotherapy, chemotherapy resistance and cardiovascular disease. We plan to optimize these compounds and conduct preclinical experiments in 2018 to further

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characterize the effects of our galectin-3 inhibitors on immune processes and anti-fibrotic activity. We are also designing other galectin inhibitors that we believe could be used to treat various diseases.

Our Collaboration with Pfizer for Rivipansel

Overview

In October 2011, we entered into a license agreement with Pfizer, under which we granted Pfizer an exclusive worldwide license to develop and commercialize rivipansel, for all fields and uses. The products licensed under the agreement also include certain backup compounds, along with modifications of and improvements to rivipansel that meet defined chemical properties.

Under the terms of the agreement, we received a $22.5 million upfront payment and are eligible to earn up to $115.0 million upon the achievement of specified development milestones, including the dosing of the first patients in Phase 3 clinical trials for up to two indications and the first commercial sale of a licensed product in the United States and selected European countries for up to two indications, up to $70.0 million upon the achievement of specified regulatory milestones, including the acceptance of our filings for regulatory approval by regulatory authorities in the United States and Europe for up to two indications, and up to $135.0 million upon the achievement of specified levels of annual net sales of licensed products. We are also eligible to receive tiered royalties for each licensed product, with percentages ranging from the low double digits to the low teens, based on net sales of rivipansel worldwide, subject to reductions in specified circumstances. The first potential milestone payment under the Pfizer agreement was $35.0 million upon the initiation of dosing of the first patient in a Phase 3 trial of rivipansel by Pfizer. Under the collaboration, Pfizer made a $15.0 million non-refundable payment to us in May 2014, and the dosing of the first patient in the Phase 3 clinical trial triggered the remaining $20.0 million milestone payment to us, which we received in August 2015. There were no payments from Pfizer received in 2016 or 2017.

Development and Commercialization Obligations

Pfizer will initially develop and seek approval for rivipansel in the field of sickle cell disease under the agreement. We were responsible for completion of the Phase 2 clinical trial relating to VOC associated with sickle cell disease. Following the completion of the Phase 2 clinical trial, we now have no further development or commercialization obligations, and Pfizer is required to use commercially reasonable efforts, at its expense, to develop, obtain regulatory approval for and commercialize rivipansel for sickle cell disease in the United States. Pfizer generally must notify us in writing promptly of any decision to cease development activities, efforts to obtain regulatory approval or commercialization of rivipansel for the first approved indication.

Governance

The agreement establishes a non-voting, joint steering committee to facilitate the exchange of information regarding the development of licensed products and the initial commercialization plans for such products.

Exclusivity Restrictions

During the term of the agreement, we may not directly or indirectly commercialize any pharmaceutical compound or product that is labeled for the treatment, prevention or prophylaxis of a vaso-occlusive or painful crisis associated with sickle cell disease anywhere in the world, subject to specified exceptions if we or our affiliates were to undergo a change of control.

Term and Termination

The agreement will expire on a licensed product-by-licensed product and country-by-country basis on the date of termination of the applicable royalty term with respect to each licensed product in each country and in its entirety upon the expiration of all applicable royalty terms for all licensed products in all countries. The royalty term for each licensed product in each country is the period commencing with the first commercial sale in the applicable country and ending on the expiration of specified patent coverage or 10 years following the first commercial sale in the applicable country, whichever is later. Pfizer has the right to terminate the agreement, subject to certain notice requirements. The agreement

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may also be terminated in its entirety either by Pfizer or by us in the event of an uncured material breach by the other party or in the event the other party becomes subject to specified bankruptcy, insolvency or similar circumstances.

Effects of Termination

Upon termination of the agreement by Pfizer for convenience or by us, all rights and licenses granted to Pfizer under the agreement will terminate and Pfizer is obligated to grant us a non-exclusive worldwide license to specified Pfizer proprietary rights to develop and commercialize licensed products in the form being used or sold by Pfizer at the time of such termination, to transfer to us specified data and regulatory materials and approvals, and to provide for the continued supply of licensed products subject to specified terms. If Pfizer has completed additional clinical trials for the applicable licensed product and we obtain such a license or obtain such data and materials and commercialize a licensed product, then, for a period of 10 years from the first commercial sale of such licensed product, Pfizer is eligible to receive royalties at defined percentages in the low single-digits on net sales of such licensed product worldwide, up to a defined aggregate payment cap. The applicable royalty rate and maximum royalty payment cap depend on the stage of clinical development at the time of such termination.

Research Services Agreement with University of Basel

We entered into a research services agreement with the University of Basel, or the University, for the discovery and evaluation of selectin antagonists. The research under this agreement has been completed; however, certain patents covering the rivipansel compound remain subject to provisions of the research services agreement. Under the terms of the research services agreement, if we receive any future milestone payments or royalties from Pfizer with respect to rivipansel, we agreed to pay the University 10% of those amounts, subject to specified exceptions. In February 2016, we paid $2.0 million to the University based upon our receipt of a $20.0 million non-refundable milestone payment from Pfizer in August 2015. We recorded this payment during the year ended December 31, 2015, at the time the payment became due to the University. There were no additional payments due to the University for the years ended December 31, 2016 or 2017. The research services agreement remains in effect until we are no longer obligated to make any potential payments.

Intellectual Property

We strive to protect the intellectual property that we believe is important to our business, including seeking and maintaining patent protection intended to cover the composition of matter of our drug candidates and their methods of use. We have issued patents directed to rivipansel and methods of use that are expected to expire between 2023 and 2030. We also have issued patents which cover GMI-1271 and methods of use that are expected to expire between 2032 and 2033. In addition, we have several pending patent applications covering GMI-1271 and/or methods of using it, the last expiring of which, if issued, currently would be predicted to expire in 2037. We also rely on trade secret protection for our confidential and proprietary information and careful monitoring of such information to protect aspects of our business.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important inventions and know-how related to our business, defend and enforce our patents, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties. We also rely on know-how and continuing technological innovation to develop, strengthen and maintain our proprietary position in the field of glycomimetics.

A third party may hold intellectual property, including patent rights that are important or necessary to the development of our drug candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our drug candidates, in which case we would be required to obtain a license from these third parties. If we are not able to obtain such a license, or are not able to obtain such a license on commercially reasonable terms, our business could be materially harmed.

We plan to continue to expand our intellectual property estate by filing patent applications directed to additional glycomimetic compounds and their derivatives, compositions and formulations containing them and methods of using them. Additionally, we will seek patent protection in the United States and internationally for novel compositions of matter covering the compounds and their use in a variety of therapies.

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The patent positions of biotechnology companies like us are generally uncertain and involve complex legal, scientific and factual questions. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance, including where a reissue application is filed in relation to an issued patent to correct issues or errors arising during prosecution that may render claims of the issued patent either wholly or partially invalid or unenforceable. Consequently, we do not know whether any of our drug candidates will be protectable or remain protected by enforceable patents. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient proprietary protection from competitors. Any patents that we hold may be challenged, circumvented or invalidated by third parties.

Because patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months, and since publication of discoveries in the scientific or patent literature often lags behind actual discoveries, we cannot be certain of the priority of inventions covered by pending patent applications. Moreover, we may have to participate in interference proceedings declared by the U.S. Patent and Trademark Office, or USPTO, or a foreign patent office to determine priority of invention or in post-grant challenge proceedings, such as oppositions, that challenge priority of invention or other features of patentability. Such proceedings could result in substantial cost, even if the eventual outcome is favorable to us.

Manufacturing

We do not have any manufacturing facilities or personnel. We currently rely, and expect to continue to rely, on third parties for the manufacturing of our drug candidates for preclinical and clinical testing, as well as for commercial manufacturing if our drug candidates receive marketing approval.

In the case of rivipansel, the initial process development, manufacturing and scale-up was managed by us and performed under contract by third parties. Under our license agreement with Pfizer, responsibility for manufacturing rivipansel has now transferred to Pfizer. With respect to our other drug candidates, we anticipate continuing to manage process development, scale-up and manufacturing under contracts with third parties. For GMI-1271, we expect a significant increase in manufacturing as we scale up for our planned Phase 3 clinical trial and prepare for potential filings for marketing approval.

All of our drug candidates are small molecules and are manufactured in reliable and reproducible synthetic processes from readily available starting materials. The chemistry does not require unusual equipment in the manufacturing process. We expect to continue to develop drug candidates that can be produced cost-effectively at contract manufacturing facilities.

Commercialization

We have not yet established a sales, marketing or drug distribution infrastructure. With the exception of rivipansel, to which we have granted Pfizer exclusive commercialization rights, we generally expect to retain commercial rights in the United States for our current drug candidates, all of which are still in preclinical or early clinical development. We believe that it will be possible for us to access the U.S. market for those drug candidates through a focused, specialized sales force.

Subject to receiving marketing approvals, we expect to commence commercialization activities by building a focused sales and marketing organization in the United States to sell our drugs. We believe that such an organization will be able to target the community of physicians who are the key specialists in treating the patient populations for which our drug candidates are being developed. Outside the United States, we expect to enter into distribution and other marketing arrangements with third parties for any of our drug candidates that obtain marketing approval.

We also plan to build a marketing and sales management organization to create and implement marketing strategies for any drugs that we market through our own sales organization and to oversee and support our sales force. The responsibilities of the marketing organization would include developing educational initiatives with respect to approved drugs and establishing relationships with thought leaders in relevant fields of medicine.

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Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary drugs. While we believe that our knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

The key competitive factors affecting the success of all of our drug candidates, if approved, are likely to be their safety, efficacy, convenience, price, the level of generic competition and the availability of coverage and reimbursement from government and other third-party payors.

Rivipansel: Sickle Cell Disease

Hydroxyurea and Endari are approved as prophylactic therapies for SCD. Based on publicly available information, we are not aware of any drugs currently approved in the United States as “on-demand” (as needed) for the treatment of SCD patients experiencing an acute VOC episode. There are a number of compounds that are in Phase 2 or Phase 3 clinical development as either prophylactic or gene therapy/blood transfusion approaches to treat patients with SCD, including:

- Prophylactic Approaches: Novartis Pharmaceuticals Corporation (crizanlizumab, formerly SelG1); Baxter International (Aes-103); and Global Blood Therapeutics (voxelotor, formerly GBT440); and

- Gene Therapy/Blood Transfusion Approaches: Blue Bird Bio (Lentiglobin BB305); Sangamo Biosciences/Bioverativ (ZFN Knockout); and Bellicum Pharmaceuticals (BPX-501).

Attempts to develop a cure for SCD through gene therapy remain at an early stage of development, with significant variability observed to date in achieving target levels of anti-sickling hemoglobin. Should one or more of these prophylactic agents or gene therapy approaches be commercialized prior to rivipansel, they could reduce the number of VOC episodes each year, reducing the market opportunity for rivipansel.

GMI-1271: AML and MM

Our drug discovery, development and commercialization activities in oncology face, and will continue to face, intense competition from organizations such as pharmaceutical and biotechnology companies, as well as academic and research institutions and government agencies. As the treatment landscape for AML and MM changes, there is substantial risk that GMI-1271 might not provide additional benefit over other therapies.

The following four new therapies were approved by the FDA for the treatment of AML in 2017:

- RYDAPT® (midostaurin), an oral prescription medicine commercialized by Novartis to be used in combination with certain chemotherapy medicines to treat adults with newly diagnosed AML who have a defect in a gene called FLT3;
- IDHIFA® (enasidenib), a prescription medicine commercialized by Celgene intended to treat people with AML with an isocitrate dehydrogenase-2 (IDH2) mutation whose disease has come back or has not improved after previous treatments;
- VYXEOSTM (daunorubicin and cytarabine), commercialized by Jazz Pharmaceuticals, which is indicated for the treatment of adults with newly-diagnosed therapy-related AML (t-AML) or AML with myelodysplasia-related changes (AML-MRC); and
- Mylotarg (gemtuzumab ozogamicin), commercialized by Pfizer, which is indicated for the treatment for the treatment of newly-diagnosed CD33-positive AML in adults (in combination with daunorubicin and cytarabine) and for treatment of relapsed or refractory CD33-positive AML in adults and in pediatric patients 2 years and older as a stand-alone treatment.

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While many chemotherapies in development for hematologic malignancies will likely be complementary to GMI-1271, there are also therapies in development that could be directly competitive with GMI-1271. In particular, there are a number of CXCR4 antagonists in clinical development that target the bone marrow microenvironment in order to mobilize and sensitize cancer cells to chemotherapy, including candidates developed by Sanofi-Aventis (Mozobil), Bristol Myers Squibb (BMS-936564), NOXXON Pharma (NOX-A12), Eli Lilly (LY2510924) and BioLine RX (BL-8040).

Many of the companies against which we are competing, or against which we may compete in the future, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Government Regulation and Product Approval

Government authorities in the United States, at the federal, state and local levels, and in other countries, extensively regulate, among other things, the research, development, testing, manufacture, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, import and export of pharmaceutical products, such as those we are developing. The processes for obtaining regulatory approvals in the United States and in foreign countries, along with subsequent compliance with applicable statutes and regulations, require the expenditure of substantial time and financial resources.

United States Government Regulation

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the drug development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve pending new drug applications, or NDAs, withdrawal of an approval, imposition of a clinical hold, issuance of warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;
- performance of human clinical trials, including adequate and well-controlled clinical trials, in accordance with good clinical practices, or GCP, to establish the safety and efficacy of the proposed drug for each indication;

- submission to the FDA of an NDA;
- satisfactory completion of an FDA advisory committee review, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practices, or cGMP, and to assure that the

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facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity, as well as satisfactory completion of an FDA inspection of selected clinical sites to determine GCP compliance; and

· FDA review and approval of the NDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess potential safety and efficacy. An IND sponsor must submit the results of the preclinical studies, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. Some preclinical testing may continue even 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 clinical trial on a 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

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must continue to oversee the clinical trial while it is being conducted. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined. In Phase 1, the drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an initial indication of its effectiveness. In Phase 2, the drug typically is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage. In Phase 3, the drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the safety and efficacy of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted, at least annually, to the FDA, and more frequently if serious adverse events occur. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, 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 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 has been associated with unexpected serious harm to patients.

A sponsor may request a Special Protocol Assessment, or SPA, the purpose of which is to reach agreement with the FDA on the Phase 3 clinical trial protocol design and analysis that will form the primary basis of an efficacy claim. According to FDA's published guidance on the SPA process, a sponsor that meets the prerequisites may make a specific request for an SPA and provide information regarding the design and size of the proposed clinical trial. The

FDA is supposed to evaluate the protocol within 45 days of the request to assess whether the protocol design and planned analysis of the trial are acceptable to support regulatory approval of the product candidate with respect to effectiveness of the indication studied, and that evaluation may result in discussions and a request for additional information. An SPA request must be made before the proposed trial begins, and all open issues must be resolved before the trial begins for an

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SPA to be approved. If a written agreement is reached, it will be documented in an SPA letter or the minutes of a meeting between the sponsor and the FDA and made part of the administrative record.

Even if the FDA agrees to the design, execution and analyses proposed in protocols reviewed under the SPA process, the FDA may revoke or alter its agreement under the following circumstances:

- public health concerns emerge that were unrecognized at the time of the protocol assessment, or the director of the review division determines that a substantial scientific issue essential to determining safety or efficacy has been identified after testing has begun;
- a sponsor fails to follow a protocol that was agreed upon with the FDA; or
- the relevant data, assumptions, or information provided by the sponsor in a request for SPA change, are found to be false statements or misstatements, or are found to omit relevant facts.

A documented SPA may be modified, and such modification will be deemed binding on the FDA review division, except under the circumstances described above, if the FDA and the sponsor agree in writing to modify the protocol and such modification is intended to improve the study. An SPA, however, does not guarantee that a trial will be successful.

Marketing Approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has agreed to certain performance goals regarding the timing of its review of an application.

In addition, under the Pediatric Research Equity Act, an NDA or supplement to an NDA must contain data that are adequate to assess the safety and effectiveness 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, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

The FDA also may require submission of a risk evaluation and mitigation strategy, or REMS, plan to mitigate any identified or suspected serious risks. The REMS plan could include medication guides, physician communication plans, assessment plans and elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission, before accepting them for filing, to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity.

The FDA typically refers questions regarding novel drugs to an external advisory committee. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

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Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical trial sites to assure compliance with GCP.

The testing and approval process for an NDA requires substantial time, effort and financial resources, and takes several years to complete. Data obtained from preclinical and clinical testing are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval of an NDA on a timely basis, or at all.

After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA’s satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

Special FDA Expedited Review and Approval Programs

The FDA has various programs, including fast track designation, accelerated approval, priority review, and breakthrough therapy designation, which are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. The FDA may review sections of the NDA for a fast track product on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

The FDA may give a priority review designation to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. A priority review means that the goal for the FDA to review an application is six months, rather than the standard review of ten months under current PDUFA guidelines. These six and ten month review periods are measured from the “filing” date rather than the receipt date for NDAs for new molecular entities, which typically adds approximately two months to the timeline for review and decision from the date of submission. Most products that are eligible for fast track designation are also likely to be considered appropriate to receive a priority review.

In addition, products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may be eligible for accelerated approval and may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured

earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require a sponsor of a drug receiving accelerated approval to perform post-marketing studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint, and the drug may be subject to accelerated withdrawal procedures.

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A sponsor can also request designation of a product candidate as a "breakthrough therapy." A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Drugs designated as breakthrough therapies are also eligible for accelerated approval. The FDA must take certain actions, such as holding timely meetings and providing advice, intended to expedite the development and review of an application for approval of a breakthrough therapy.

Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. We may explore some of these opportunities for our product candidates as appropriate.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications, manufacturing changes or other labeling claims, are subject to further testing requirements and prior FDA review and approval. There also are continuing annual user fee requirements for any marketed products, as well as application fees for supplemental applications with clinical data.

Even if the FDA approves a product, it may limit the approved indications for use of the product, require that contraindications, warnings or precautions be included in the product labeling, including a boxed warning, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms under a REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

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

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The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Although physicians, in the practice of medicine, may prescribe approved drugs for unapproved indications, pharmaceutical companies generally are required to promote their drug products only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Federal and State Fraud and Abuse, Data Privacy and Security, and Transparency Laws and Regulations

In addition to FDA restrictions on marketing of pharmaceutical products, federal and state healthcare laws and regulations restrict business practices in the biopharmaceutical industry. These laws include anti-kickback and false claims laws and regulations, data privacy and security, and transparency laws and regulations.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term “remuneration” has been broadly interpreted to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers and formulary managers on the other. Although there are a number of statutory exemptions and regulatory safe harbors protecting some common activities from prosecution, the exemptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Several courts have interpreted the statute’s intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the statute has been violated.

The reach of the federal Anti-Kickback Statute was also broadened by the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively PPACA, which, among other things, amended the intent requirement of the federal Anti-Kickback Statute such that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, PPACA provides that the government may assert that 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 federal civil False Claims Act or the civil monetary penalties statute, which imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Federal false claims laws, including the federal civil False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. A claim includes “any request or demand” for money or property presented to the U.S. government. Several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies’ marketing of products for unapproved, and thus non-reimbursable, uses. The federal Health Insurance Portability and

Accountability Act of 1996, or HIPAA, created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

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In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their respective implementing regulations, including the Final HIPAA Omnibus Rule published on January 25, 2013, imposes specified requirements on certain types of individuals and entities relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's security standards directly applicable to "business associates," defined as independent contractors or agents of covered entities that create, receive, maintain or transmit protected health information in connection with providing a service for or on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, 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 attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in certain circumstances, many of which are not pre-empted by HIPAA, differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other transfers of value made to physicians and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members.

We may also be subject to state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, as well as state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exemptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including criminal and significant civil monetary penalties, damages, fines, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, disgorgement, exclusion from participation in government healthcare programs and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

Coverage and Reimbursement

The future commercial success of our drug candidates or any of our collaborators' ability to commercialize any approved drug candidates successfully will depend in part on the extent to which governmental payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers and other third-party payors provide coverage for and establish adequate reimbursement levels for our drug candidates. Government health administration authorities, private health insurers and other organizations generally decide which drugs they will pay for and establish reimbursement levels for healthcare. In particular, in the United States, private health insurers and other

third-party payors often provide reimbursement for products and services based on the level at which the government, through the Medicare or Medicaid programs, provides reimbursement for such treatments. In the United States, the EU and other potentially significant markets for our drug candidates, government authorities and third party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which often has resulted in average selling prices lower than they would otherwise be. Further, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care

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groups, judicial decisions and laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical coverage and reimbursement policies and pricing in general.

Third-party payors are increasingly imposing additional requirements and restrictions on coverage and limiting reimbursement levels for medical products. For example, federal and state governments reimburse covered prescription drugs at varying rates generally below average wholesale price. These restrictions and limitations influence the purchase of healthcare services and products. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drug products for a particular indication. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our drug candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in drug development. Legislative proposals to reform healthcare or reduce costs under government insurance programs may result in lower reimbursement for our drugs and drug candidates or exclusion of our drugs and drug candidates from coverage. The cost containment measures that healthcare payors and providers are instituting and any healthcare reform could significantly reduce our revenues from the sale of any approved drug candidates. We cannot provide any assurances that we will be able to obtain and maintain third party coverage or adequate reimbursement for our drug candidates in whole or in part.

Impact of Healthcare Reform on our Business

The United States and some foreign jurisdictions are considering enacting or have enacted a number of additional legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts, which include major legislative initiatives to reduce the cost of care through changes in the healthcare system, including limits on the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

There have been several U.S. government initiatives over the past few years to fund and incentivize certain comparative effectiveness research, including creation of the Patient-Centered Outcomes Research Institute under PPACA. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of any product, if any such product or the condition that it is intended to treat is the subject of a study. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's product could adversely affect the sales of our product candidates. If third-party payors do not consider our drug candidates to be cost-effective compared to other available therapies, they may not cover our drug candidates, once approved, as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our drugs on a profitable basis. PPACA became law in March 2010 and substantially changed the way healthcare is financed by both governmental and private insurers. Among other measures that may have an impact on our business, PPACA establishes an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; a new Medicare Part D coverage gap discount program; and a new formula that increases the rebates a manufacturer must pay under the Medicaid Drug Rebate Program. Additionally, PPACA extends manufacturers' Medicaid rebate liability, expands eligibility criteria for Medicaid programs, and expands entities eligible for discounts under the Public Health Service

pharmaceutical pricing program. At this time, we are unsure of the full impact that PPACA will have on our business. There have been judicial and Congressional challenges to certain aspects of PPACA, as well as recent efforts by the Trump administration to repeal or replace certain aspects of the PPACA, and we expect such challenges and amendments to continue.

Since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of any certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of

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certain taxes under the PPACA have been signed into law. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the PPACA 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". Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share and the medical device excise tax on non-exempt medical devices.

In addition, 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 proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

As a result of PPACA, Medicare payments are increasingly tied to quality of care and value measures, and reporting of related data by providers such as physicians and hospitals. So called "value based reimbursement" measures may present challenges as well as potential opportunities for biopharmaceutical manufacturers. Medicare incentives for providers meeting certain quality measures may ultimately prove beneficial for manufacturers that are able to establish that their products may help providers to meet such measures. However, manufacturers' ability to market their drug products based on quality or value is highly regulated and not always permissible. In addition, the potentially decreased Medicare reimbursement to those providers that fail to adequately comply with quality reporting requirements could translate to decreased resources available to purchase products and may negatively impact marketing or utilization of our drug candidates if they are approved for marketing. We cannot predict at this time what impact, if any, the longer-term shift towards value based reimbursement will have on any of our drug candidates in either the Medicare program, or in any other third party payor programs that may similarly tie payment to provider quality.

In addition, other legislative changes have been proposed and adopted since PPACA was enacted. In August 2011, the President signed into law the Budget Control Act of 2011, as amended, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee on Deficit Reduction did not achieve its targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which began in 2013 and, following passage of the Bipartisan Budget Act of 2015, will continue through 2025 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These and other healthcare reform initiatives may result in additional reductions in Medicare and other healthcare funding.

Exclusivity and Approval of Competing Products

Hatch-Waxman Patent Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims that cover the applicant's product or a method of using the product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an abbreviated new drug application, or ANDA, or 505(b)(2) NDA. Generally, an ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths, dosage form and route of administration as the listed drug and has been shown to be bioequivalent through in vitro or in vivo testing or otherwise to the listed drug. ANDA applicants are not required to conduct or submit results of preclinical or clinical tests to prove the safety or efficacy of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often

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be substituted by pharmacists under prescriptions written for the original listed drug. 505(b)(2) NDAs generally are submitted for changes to a previously approved drug product, such as a new dosage form or indication.

The ANDA or 505(b)(2) NDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval. Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

Generally, the ANDA or 505(b)(2) NDA cannot be approved until all listed patents have expired, except when the ANDA or 505(b)(2) NDA applicant challenges a listed drug. A certification that the proposed product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicate that it is not seeking approval of a patented method of use, the ANDA or 505(b)(2) NDA application will not be approved until all the listed patents claiming the referenced product have expired.

If the ANDA or 505(b)(2) NDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the application has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of notice of the Paragraph IV certification automatically prevents the FDA from approving the ANDA or 505(b)(2) NDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

Hatch-Waxman Non-Patent Exclusivity

Market and data exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications for competing products. The FDCA provides a five-year period of non-patent data exclusivity within the United States 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 activity of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company that contains the previously approved active moiety. However, an ANDA or 505(b)(2) NDA may be submitted after four years if it contains a certification of patent invalidity or noninfringement.

The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA or 505(b)(2) 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 or supplement. Three-year exclusivity may be awarded for changes to a previously approved drug product, such as new indications, dosages, strengths or dosage forms of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug product. 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.

Orphan Drug Exclusivity

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

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the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan designation must be requested before submitting an NDA or biologics license application. Orphan designation does not convey any advantage in or shorten the duration of the regulatory review and approval process. We have received orphan drug designation for rivipansel and GMI-1271, and we intend to seek orphan drug designation and exclusivity for our other drug candidates whenever it is available.

If a product that has orphan designation subsequently receives the first FDA approval for such drug for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the EU has similar, but not identical, benefits.

Pediatric Exclusivity

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan drug exclusivity periods described above. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA’s request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or Orange Book listed patent protection cover the drug are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve an ANDA or 505(b)(2) application owing to regulatory exclusivity or listed patents. If any of our drug candidates is approved, we anticipate seeking pediatric exclusivity when it is appropriate.

Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our drug candidates. For example, in the EU, we must obtain authorization of a clinical trial application, or CTA, in each member state in which we intend to conduct a clinical trial. Whether or not we obtain FDA approval for a drug, we would need to obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the drug in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

Employees

As of December 31, 2017, we had 40 full-time employees, all of whom are located in the United States. None of our employees is represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

Legal Proceedings

We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceeding against us that we believe could have a material adverse effect on our business, operating results or financial condition.

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Information about Segments

We operate only in one business segment. See “Note 2—Summary of Significant Accounting Policies—Segment Information” to our financial statements contained in Part II, Item 8 of this Annual Report.

Customer Concentration and Geographic Information

We did not have any material revenue for the years ending December 31, 2017 and 2016. Substantially all of our revenue for the year ended December 31, 2015 was derived from Pfizer and was earned in the United States. All of our long-lived assets are located in the United States.

Corporate Information

We were incorporated under the laws of the State of Delaware in April 2003 and commenced operations in May 2003. Our principal executive offices are located at 9708 Medical Center Drive, Rockville, Maryland 20850. Our telephone number is (240) 243-1201.

Available Information

Our internet website address is www.glycomimetics.com. In addition to the information contained in this Annual Report, information about us can be found on our website. Our website and information included in or linked to our website are not part of this Annual Report.

Our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, are available free of charge through our website as soon as reasonably practicable after they are electronically filed with or furnished to the Securities and Exchange Commission, or SEC. The public may read and copy the materials we file with the SEC at the SEC’s Public Reference Room at 100 F Street, NE, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. Additionally the SEC maintains an internet site that contains reports, proxy and information statements and other information. The address of the SEC’s website is www.sec.gov.

ITEM 1A.RISK FACTORS

Our business is subject to numerous risks. You should carefully consider the following risks, as well as general economic and business risks, and all of the other information contained in this Annual Report, together with any other documents we file with the SEC. Any of the following risks could have a material adverse effect on our business, operating results and financial condition and cause the trading price of our common stock to decline.

Risks Related to Our Financial Position and Capital Needs

We have incurred significant losses since our inception. We expect to continue to incur losses over the next several years and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. As of December 31, 2017, we had an accumulated deficit of $152.3 million. We have financed our operations to date with $64.1 million raised in private placements of convertible debt and convertible preferred stock, an aggregate of $57.5 million received from upfront and milestone payments under our license agreement with Pfizer and $194.5 million from registered public offerings of our common stock. We have not generated any meaningful revenue since our inception other than from the upfront and milestone payments from Pfizer.

We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials. We are still in the early stages of development of our drug candidates, and we have not completed development of any drugs. We expect to continue to incur significant expenses and operating losses over

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the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. Although responsibility for further development, regulatory approval and potential commercialization of our first drug candidate, rivipansel, has transferred to Pfizer under our collaboration with them following the completion of our Phase 2 clinical trial, we anticipate that our expenses will increase substantially as we:

- conduct clinical trials of GMI-1271 in AML and MM;
- conduct clinical trials of GMI-1359;
- continue the research and development of our other drug candidates;
- seek to discover and develop additional drug candidates;
- seek regulatory approvals for any drug candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any drugs other than rivipansel for which we may obtain regulatory approval;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, quality control and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our drug development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses in operating as a public company.

To become and remain profitable, we must succeed in developing and eventually commercializing drugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our drug candidates other than rivipansel, obtaining regulatory approval for these drug candidates and manufacturing and commercializing any drugs for which we may obtain regulatory approval, as well as discovering additional drug candidates. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenue that is significant enough to achieve profitability.

In the case of rivipansel, our ability to generate revenue is dependent upon the achievement of development, regulatory and commercial milestones and sales sufficient to generate royalties under our license agreement with Pfizer, and the achievement of such milestones is largely out of our control. If Pfizer fails, or chooses not to continue, to further develop, seek regulatory approval for or commercialize rivipansel, our ability to generate revenue with respect to rivipansel will be significantly reduced or eliminated. Because all of our drug candidates other than rivipansel are still in preclinical or early clinical development, if we are unable to generate revenue from our license agreement with Pfizer, we may never become profitable, and we may not be able to invest in the further development of our other drug candidates.

Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. If we are required by regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in completing our clinical trials or the development of any of our drug candidates, our expenses could increase.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will need substantial additional funding to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our drug development programs or potential commercialization efforts.

We believe that our cash and cash equivalents as of December 31, 2017 will enable us to fund our operating expenses and capital expenditure requirements at least through the end of 2019, without giving effect to any potential

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milestone payments we may receive under our agreement with Pfizer. However, we will need to obtain substantial additional funding in connection with our continuing operations. Our future capital requirements will depend on many factors, including:

- our agreement with Pfizer remaining in effect and our ability to achieve milestones under this and any other license or collaboration agreement that we may enter into in the future;
- the progress and results of the Phase 3 clinical trial of rivipansel;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other drug candidates, including our ongoing and planned clinical trials of GMI-1271 and GMI-1359;
- the number and development requirements of other drug candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our drug candidates;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our drug candidates other than rivipansel for which we receive marketing approval;
- any royalties we receive from Pfizer with respect to sales of rivipansel, if it receives marketing approval;
- the revenue, if any, received from commercial sales of our drug candidates other than rivipansel for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims; and
- the extent to which we acquire or in-license other drug candidates and technologies.

Identifying potential drug candidates and conducting preclinical testing and clinical trials is a time consuming, expensive and uncertain process that takes years to complete, and we and Pfizer or any future collaborators may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our drug candidates, if approved, may not achieve commercial success. Our commercial revenue, if any, will be derived from the sale of drugs that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our drug candidates.

Until such time, if ever, as we can generate substantial revenue from the sale of our drugs, we expect to finance our cash needs through a combination of equity offerings, debt financings and license and development agreements. We do not currently have any committed external source of funds other than possible milestone payments and possible royalties under our license agreement with Pfizer. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your 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 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 be required to relinquish valuable rights to our research programs or drug 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 with third parties when needed, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to third parties to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

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Our operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2003, and our operations to date have been largely focused on raising capital, developing our expertise in carbohydrate chemistry and knowledge of carbohydrate biology, identifying potential drug candidates, undertaking preclinical studies and conducting clinical trials. We have three drug candidates in clinical development, but we have not yet demonstrated our ability to successfully complete later stage clinical trials, obtain regulatory approvals, manufacture a commercial scale drug, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. With respect to our drug candidates other than rivipansel, we will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law new legislation that significantly revises the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the new federal tax law is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the newly enacted federal tax law. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

Our ability to use net operating losses to offset future taxable income may be subject to limitations.

As of December 31, 2017, we had federal and state net operating loss carryforwards of $112.0 million. The federal and state net operating loss carryforwards will begin to expire, if not utilized, beginning in 2026. These net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the newly enacted federal income tax law, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various states will conform to the newly enacted federal tax law. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. We could experience ownership changes in the

future that would limit our ability to use our net operating loss carryforwards.

Risks Related to the Discovery and Development of Our Drug Candidates

Our research and development is focused on discovering and developing novel glycomimetic drugs, and we are taking an innovative approach to discovering and developing drugs, which may never lead to marketable drugs.

A key element of our strategy is to use and expand our platform to build a pipeline of novel glycomimetic drug candidates and progress these drug candidates through clinical development for the treatment of a variety of diseases. The discovery of therapeutic drugs based on molecules that mimic the structure of carbohydrates is an emerging field,

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and the scientific discoveries that form the basis for our efforts to discover and develop drug candidates are relatively new. The scientific evidence to support the feasibility of developing drug candidates based on these discoveries is both preliminary and limited. Although our research and development efforts to date have resulted in a pipeline of glycomimetic drug candidates, we may not be able to develop drug candidates that are safe and effective. Even if we are successful in continuing to build our pipeline, the potential drug candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize drug candidates based upon our glycomimetics platform, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

We are very early in our development efforts and have only three drug candidates that are in clinical trials. All of our other drug candidates are still in preclinical development. If we or our collaborators are unable to commercialize our drug candidates or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and rivipansel, GMI-1271 and GMI-1359 are our only drug candidates that are in clinical trials. Our other drug candidates are still in preclinical development. We have not completed the development of any drug candidates, we currently generate no revenue from the sale of any drugs and we may never be able to develop a marketable drug. We have invested substantially all of our efforts and financial resources in the development of our glycomimetics platform, the identification of potential drug candidates using that platform and the development of our drug candidates. Other than with respect to rivipansel, for which our collaborator Pfizer now has the responsibility for further development and commercialization, our ability to generate revenue from our other drug candidates, which we do not expect will occur for many years, if ever, will depend heavily on their successful development and eventual commercialization. The success of those drug candidates will depend on several factors, including:

- successful completion of preclinical studies and clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our drug candidates;
- making arrangements with third-party manufacturers for, or establishing, commercial manufacturing capabilities;
- launching commercial sales of the drugs, if and when approved, whether alone or in collaboration with others;
- acceptance of the drugs, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- obtaining and maintaining healthcare coverage and adequate reimbursement;
- protecting our rights in our intellectual property portfolio; and
- maintaining a continued acceptable safety profile of the drugs following approval.

If we do not achieve 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 drug candidates, which would materially harm our business.

Clinical drug 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 our drug candidates.

All but three of our drug candidates are in preclinical development, and their risk of failure is high. It is impossible to predict when or if any of our drug candidates will prove safe or effective in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any drug candidate, we or a collaborator must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety

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and efficacy of the drug candidate in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of development. The outcome of preclinical testing 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 drug candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs.

We or our current or future collaborators may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our or their ability to receive marketing approval or commercialize our drug candidates, including:

- regulators or institutional review boards 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 clinical trial contracts or clinical trial protocols with prospective trial sites;
- clinical trials of our drug candidates may produce negative or inconclusive results, including failure to demonstrate statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs;
    - the number of patients required for clinical trials of our drug 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 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;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our drug candidates may be greater than we anticipate;
- the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates may be insufficient or inadequate; and
- our drug candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials.

If we are required to conduct additional clinical trials or other testing of our drug candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our drug candidates or other testing, if the results of these clinical trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our drug candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements; or
- have the drug removed from the market after obtaining marketing approval.

Our drug development costs will also increase if we experience delays in 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 study or clinical trial delays also could shorten any

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periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring drugs to market before we do, and thereby impair our ability to successfully commercialize our drug candidates.

If we or our collaborators experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We or our collaborators may not be able to initiate or continue clinical trials for our drug candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States. In particular, because our lead drug candidates are intended to treat patients with orphan diseases such as sickle cell disease, AML and MM, our or our collaborators' ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate. In addition, some of our competitors have ongoing clinical trials for drug candidates that treat the same or similar indications as our drug candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' drug candidates. Patient enrollment is also affected by other factors, including:

- the severity of the disease or condition under investigation;
- the eligibility criteria for the trial;
- the perceived risks and benefits of the drug candidate;
- the availability of drugs approved to treat the disease or condition under investigation;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our or our collaborators' inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us or them to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our drug candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

If serious adverse or unacceptable side effects are identified during the development of our drug candidates, we may need to abandon or limit the development of some of our drug candidates.

If our drug candidates are associated with undesirable side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit their development to more narrow uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many drug candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented their further development.

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

Because we have limited financial and management resources, we focus on a limited number of research programs and drug candidates. As a result, we may forego or delay pursuit of opportunities with other drug 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 drugs or profitable market opportunities. Our spending on current and future research and development programs and drug candidates for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other

arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights.

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Risks Related to Our Dependence on Third Parties

Our success is highly dependent on our existing collaboration with Pfizer, and future collaborations may also be important to us. If we are unable to maintain any of these collaborations, or if these collaborations are not successful, our business could be adversely affected.

We have limited capabilities for drug development and do not yet have any capabilities for sales, marketing or distribution. Under our license agreement with Pfizer, Pfizer is responsible for all further development, regulatory approval and potential commercialization efforts with respect to rivipansel. Other than rivipansel, GMI-1271 and GMI-1359, all of our drug candidates are still in preclinical development, and therefore our success is highly dependent on our collaboration with Pfizer. We cannot assure you that Pfizer will continue to develop rivipansel in a timely manner, or at all, or, if it achieves regulatory approval, that Pfizer will successfully commercialize rivipansel.

Our Pfizer collaboration, and any future collaborations we might enter into, may pose a number of risks, including:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue the commercialization of any drug candidates that achieve regulatory approval or may elect not to pursue, continue or renew development or commercialization of drug candidates based on clinical trial results, changes in such collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;
- collaborators could experience delays in initiating or conducting clinical trials for any number of reasons;
- collaborators could independently develop, or develop with third parties, drugs that compete directly or indirectly with our drugs or drug candidates if such 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;
- drug candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own drug candidates or drugs, which may cause such collaborators to cease to devote resources to the commercialization of our drug candidates;
- a collaborator with marketing and distribution rights to one or more of our drug candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such drug or drugs;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of drug candidates, might lead to additional responsibilities for us with respect to drug candidates or might result in litigation or arbitration, any of which would be time consuming and expensive;
- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable drug candidates.

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If our collaboration with Pfizer or any other collaborations we might enter into in the future do not result in the successful development and commercialization of drugs, 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. In addition, even if we are eligible to receive these payments, they could be substantially delayed. For example, under our license agreement, Pfizer has the option to commence another Phase 2 clinical trial of rivipansel, and such commencement would delay or inhibit our ability to receive some of the milestone payments we might otherwise have received under the agreement. If we do not receive the funding we expect under these agreements, the development of our drug candidates could be delayed and we may need additional resources to develop our drug candidates. All of the risks relating to drug development, regulatory approval and commercialization described in this report also apply to the activities of our collaborators.

If Pfizer or a future collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate development or commercialization of any drug candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our reputation in the business and financial communities could be adversely affected. For our drug candidates other than rivipansel, we may in the future determine to collaborate with pharmaceutical and biotechnology companies for their development and potential commercialization. We face significant competition in seeking appropriate collaborators. Our ability to reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of a collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a drug candidate, reduce or delay its development 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 fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our drug candidates or bring them to market, which would impair our business prospects.

We expect to rely on third parties to conduct our future clinical trials for drug candidates other than rivipansel, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We currently expect to engage a third-party contract research organization, or CRO, to conduct our ongoing and planned clinical trials for GMI-1271 and GMI-1359 and any of our other drug candidates that may progress to clinical development. We expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials. Agreements with such third parties might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that would delay our drug development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities, but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and significant

civil and criminal sanctions.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our drug candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our drug candidates.

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We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our drug candidates or commercialization of our drugs, producing additional losses and depriving us of potential revenue.

We contract with third parties for the manufacturing of some of our drug candidates for preclinical and clinical testing and expect to continue to do so for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs, or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities or personnel. For our drug candidates other than rivipansel, for which manufacturing responsibility has shifted to Pfizer, we rely, and expect to continue to rely, on third parties for the manufacturing of our drug candidates for preclinical and clinical testing, as well as for commercial manufacture if any of our drug candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our drug candidates or drugs, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our ability to timely conduct our clinical trials or our other development or commercialization efforts.

We also expect to rely on third-party manufacturers or third-party collaborators for the manufacturing of commercial supply of any other drug candidates for which we or our collaborators obtain marketing approval. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the manufacturing agreement by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how; and
- the possible termination or non-renewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with current good manufacturing practices, or cGMP, regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of drug candidates or drugs, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our drugs.

In addition, in the event that any of our third-party manufacturers fails to comply with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third party, which we may not be able to do on commercially reasonable terms, if at all. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. If our current contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may incur added costs and delays in identifying and qualifying any such replacement. Any replacement of our manufacturers could require significant effort and expertise because there may be a limited number of qualified replacements. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations and guidelines. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop our drug candidates in a timely manner or within budget.

Our current and anticipated future dependence upon others for the manufacturing of our drug candidates or drugs may adversely affect our future profit margins and our ability to commercialize any drugs that receive marketing approval on a timely and competitive basis.

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We, or our third-party manufacturers, may be unable to successfully scale-up manufacturing of our drug candidates in sufficient quality and quantity, which would delay or prevent us from conducting our ongoing and planned clinical trials and developing our drug candidates.

In order to conduct our ongoing and planned clinical trials of our drug candidates, we will need to manufacture them in large quantities. We, or our manufacturing partners, may be unable to successfully increase the manufacturing capacity for any of our drug candidates in a timely or cost-effective manner, or at all. In addition, quality issues may arise during scale-up activities. If we or our manufacturing partners are unable to successfully scale up the manufacture of our drug candidates in sufficient quality and quantity, the development, testing and clinical trials of that drug candidate may be delayed or become infeasible, and marketing approval or commercial launch of any resulting drug may be delayed or not obtained, which could significantly harm our business.

Risks Related to the Commercialization of Our Drug Candidates

Even if any of our drug candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our drug candidates receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our drug candidates do not achieve an adequate level of acceptance, we may not generate significant revenue from drug sales and we may not become profitable. The degree of market acceptance of our drug candidates, if approved for commercial sale, will depend on a number of factors, including:

· the efficacy and potential advantages compared to alternative treatments;
· our ability to offer our drugs for sale at competitive prices;
· the convenience and ease of administration compared to alternative treatments;
· the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
· the strength of marketing and distribution support;
· the availability of third-party coverage and adequate reimbursement;
· the prevalence and severity of any side effects; and
· any restrictions on the use of our drugs together with other medications.

If we are unable to establish sales, marketing and distribution capabilities for drug candidates other than rivipansel, we may not be successful in commercializing those drug candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical drugs. Under our collaboration with Pfizer, Pfizer is responsible for the commercialization of rivipansel, our first drug candidate, if it receives regulatory approval. To achieve commercial success for any other drug candidate for which we may obtain marketing approval, we will need to establish a sales and marketing organization to market or co-promote such drugs. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a drug candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our drugs on our own include:

· our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
·

the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future drugs;

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- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more products; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and enter into arrangements with third parties to perform these services, our revenue and our profitability, if any, are likely to be lower than if we were to sell, market and distribute any drugs that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our drug candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our drugs effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our drug candidates.

We face substantial competition, which may result in others discovering, developing or commercializing drugs before or more successfully than we do.

The development and commercialization of new drugs is highly competitive. We face competition with respect to our current drug candidates, and we will face competition with respect to any drug candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, academic institutions, governmental agencies and public and private research institutions. Should any competitors' drug candidates receive regulatory or marketing approval prior to ours, they may establish a strong market position and be difficult to displace or diminish the need for our drug candidates.

The key competitive factors affecting the success of all of our drug candidates, if approved, are likely to be their safety, efficacy, convenience, price, the level of generic competition and the availability of coverage and reimbursement from government and other third-party payors. As described above under "Business—Competition," we expect that both rivipansel and GMI-1271 will compete with approved therapies and those currently in development by other companies. To the extent that competitive drugs or drug candidates developed by others are successful in treating our target indications, it could reduce the market opportunity for our drug candidates.

Many of the companies against which we are competing, or against which we may compete in the future, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize drugs that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any drugs that we may develop. Our competitors also may obtain FDA or other regulatory approval for their drugs more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

In addition, because we have no patents with respect to our glycomimetics platform, our competitors may use our methods, or acquire similar expertise, in order to develop glycomimetic drug candidates and progress these drug

candidates through clinical development and commercialization, which could impair our ability to successfully commercialize our drug candidates or otherwise limit our commercial opportunities.

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Even if we or our collaborators are able to commercialize any of our drug candidates, the drugs may become subject to unfavorable pricing regulations or third-party coverage and reimbursement policies.

Our and our collaborators' ability to commercialize any of our drug candidates successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these drugs and related treatments will be available from government payor programs at the federal and state levels authorities, including Medicare and Medicaid, private health insurers, managed care plans 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. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs. Coverage and reimbursement may not be available for any drug that we or our collaborators commercialize and, even if these are available, the level of reimbursement may not be satisfactory. Inadequate reimbursement levels may adversely affect the demand for, or the price of, any drug candidate for which we or our collaborators obtain marketing approval. Obtaining and maintaining adequate reimbursement for our drugs may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we or our collaborators may not be able to successfully commercialize any drug candidates for which marketing approval is obtained.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for coverage and reimbursement does not imply that a drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new drugs, 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 drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs 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 drugs 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. However, one payor's determination to provide coverage for a drug does not assure that other payors will also provide coverage for the drug. Our or our collaborators' inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved drugs that we develop could adversely affect our operating results, our ability to raise capital needed to commercialize drugs and our overall financial condition.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drugs vary widely from country to country. Current and future 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 drug before it can be marketed. In many countries, the pricing review period begins after marketing or 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 or our collaborators might obtain marketing approval for a drug in a particular country, but then be subject to price regulations that delay commercial launch of the drug, possibly for lengthy time periods, and negatively impact our ability to generate revenue from the sale of the drug in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more drug candidates, even if our drug candidates obtain marketing approval.

There can be no assurance that our drug candidates, if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary for a specific indication, that they will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell our drug candidates profitably if they are approved for sale.

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Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any drugs that we may develop.

We face an inherent risk of product liability exposure related to the testing of our drug candidates in human clinical trials, and will face an even greater risk if we commercially sell any drugs that we may develop. If we cannot successfully defend ourselves against claims that our drug candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any drug candidates or drugs that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any drugs that we may develop.

We currently hold $5.0 million of clinical trial insurance coverage in the aggregate for our clinical trials being conducted in the United States, with a per incident limit of $5.0 million, which may not be adequate to cover all liabilities that we may incur. In addition, we have increased our clinical coverage above this amount in foreign countries where we plan to have sites as part of our clinical trials for GMI-1271, including Ireland, Australia, Denmark, Germany and England. We may need to further increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our drug candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain patent protection for our drug candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize drug candidates similar or identical to ours, and our ability to successfully commercialize our drug candidates may be impaired.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our drug candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our drug candidates.

The patent prosecution process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know with certainty whether 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. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our drug candidates, in whole or in part, or which effectively prevent others from commercializing competitive drug candidates.

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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.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The U.S. Patent and Trademark Office recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Moreover, we may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our drug candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative drug candidates in a non-infringing manner.

In addition, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical drug candidates, or limit the duration of the patent protection of our drug candidates. Given the amount of time required for the development, testing and regulatory review of new drug 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 sufficient rights to exclude others from commercializing drugs similar or identical to ours.

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 issued patents 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. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent’s claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly.

We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent, rights that are important or necessary to the development of our drug candidates. It may be necessary for us to use patented or proprietary technology of third parties to commercialize our drug candidates, in which case we would be required to obtain a license from these third parties on commercially reasonable terms, or our business could be harmed, possibly materially.

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Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability, and the ability of our collaborators, to develop, manufacture, market and sell our drug candidates without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our drug candidates, including interference or derivation proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our drug candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing drug. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our drug candidates or force us to cease some of our business operations. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these employees or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or 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 prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially

increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

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

In addition to seeking patents for our drug candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. For example, our platform is based on trade secrets that consist largely of expertise in carbohydrate chemistry and knowledge of carbohydrate biology. We do not believe that we can obtain patent protection for our platform. Thus, our competitors may use our methods, or acquire similar expertise, in order to develop glycomimetic drug candidates and progress these drug candidates through clinical development and commercialization, which could impair our ability to successfully commercialize our drug candidates.

We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, 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 any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Risks Related to Regulatory Approval of Our Drug Candidates and Other Legal Compliance Matters

If we or our collaborators are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we or they will not be able to commercialize our drug candidates and our ability to generate revenue will be materially impaired.

Our drug candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the European Medicines Agency, or EMA, and similar regulatory authorities outside the United States. Failure to obtain marketing approval for a drug candidate will prevent us or our collaborators from commercializing the drug candidate. We have not received approval to market any of our drug candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CROs to assist us in this process for drug candidates other than rivipansel. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the drug candidate’s safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, applicable regulatory authorities. Our drug candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our ability to obtain marketing approval or prevent or limit commercial use. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the drug.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive and may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a

variety of factors, including the type, complexity and novelty of the drug candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations or changes in regulatory review for each submitted drug application may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application, or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a drug candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved drug not commercially viable.

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For marketing exclusivity in the treatment of an ongoing VOC episode in sickle cell disease, we expect to rely primarily on the orphan drug designation that the FDA has granted us for rivipansel, which includes the treatment of the complications of sickle cell disease. We have similarly received orphan drug designation for GMI-1271 from the FDA as well as from the EMA in the European Union as a potential treatment for AML. However, in order to obtain marketing exclusivity in a particular jurisdiction, we must receive the first marketing approval of the drug for its intended indication. In addition, the orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If we experience delays in obtaining approval or if we fail to obtain approval of our drug candidates, the commercial prospects for our drug candidates may be harmed and our ability to generate revenue will be materially impaired.

Even though we have obtained orphan drug designation for our most advanced drug candidate, rivipansel, and for GMI-1271, we may not be able to obtain orphan drug marketing exclusivity for these drug candidates or any of our other drug candidates.

Regulatory authorities in some jurisdictions, including the United States and the European Union, or EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. We have obtained orphan drug designation from the FDA and the EMA for rivipansel for the treatment of VOC. We have also obtained orphan drug designation from the FDA for GMI-1271 for the treatment of AML. We may seek orphan drug designation for our other drug candidates or for GMI-1271 for additional indications, such as MM. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and 10 years in the EU. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we obtain orphan drug exclusivity for a drug candidate, that exclusivity may not effectively protect the candidate from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve another drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

The FDA fast track designation for rivipansel and GMI-1271 and additional breakthrough designation for GMI-1271 may not actually lead to a faster development or regulatory review or approval process.

If a drug is intended for the treatment of a serious or life-threatening disease or condition and the drug demonstrates the potential to address unmet medical needs for this disease or condition, the drug sponsor may apply for FDA fast track designation. If fast track designation is obtained, the FDA may initiate review of sections of a new drug application, or NDA, before the application is complete. This “rolling review” is available if the applicant provides, and the FDA approves, a schedule for submission of the individual sections of the application.

Although we have obtained a fast track designation from the FDA for rivipansel to treat VOC and for GMI-1271 to treat AML and breakthrough designation for GMI-1271 to treat AML, we may not experience a faster development process, review or approval compared to conventional FDA procedures. Our fast track designation may be withdrawn

by the FDA if it believes that the designation is no longer supported by data from our clinical development programs. Our fast track designation does not guarantee that we will qualify for or be able to take advantage of the expedited review procedures or that we will ultimately obtain regulatory approval of rivipansel or GMI-1271.

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Failure to obtain marketing approval in international jurisdictions would prevent our drug candidates from being marketed abroad.

In order to market and sell our drugs in the EU and any other jurisdictions, we or our 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 drug be approved for reimbursement before it can be approved for sale in that country. We or our collaborators 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. However, failure to obtain approval in one jurisdiction may impact our ability to obtain approval elsewhere. We or our collaborators may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our drugs in any market.

A variety of risks associated with marketing our drug candidates internationally could hurt our business.

We or our collaborators may seek regulatory approval for rivipansel and our other drug candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market with low or lower prices rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations related to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our potential international operations may compromise our ability to achieve or maintain profitability.

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Any drug candidate for which we obtain marketing approval could be subject to post-marketing restrictions or recall or withdrawal from the market, and we may therefore be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our drug candidates, when and if any of them are approved.

Any drug candidate for which we obtain marketing approval, along with manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such drug candidate, 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 manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a drug candidate is granted, the approval may be subject to limitations on the indicated uses for which the drug may be marketed or to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy. If any of our drug candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit its sales.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the drug. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use, and if we do not market our drugs for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our drugs, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have negative consequences, including:

- restrictions on such drugs, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a drug;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters;
- recall or withdrawal of the drugs from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- clinical holds;
- fines, restitution or disgorgement of revenue or profit;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our drugs;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with EU requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of drugs for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the EU's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

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Our current and future business and relationships with customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

Healthcare providers and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our current and future arrangements with third-party payors and customers may expose us 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 we conduct clinical research, sell, market and distribute any drugs for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient data privacy and security regulation by the U.S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal healthcare programs, such as Medicare and Medicaid;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- 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 obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Open Payments program, pursuant to the Physician Payments Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to "payments or other transfers of value" made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members, with disclosure of such information to be made by CMS on a publicly available website; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and

state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, disgorgement, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including our collaborators, is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government healthcare programs, which could also materially affect our business.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our drug candidates and affect the prices we may obtain.

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 prevent or delay marketing approval of our drug candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any drug candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts, which include major legislative initiatives to reduce the cost of care through changes in the healthcare system, including limits on the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug. In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively PPACA, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, improve quality of care, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of PPACA of importance to our business and potential drug candidates are:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for non-compliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for a manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

· expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the federal poverty level beginning in 2014, thereby potentially increasing a manufacturer's Medicaid rebate liability;

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- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- the new requirements under the federal Open Payments program and its implementing regulations;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

There have been judicial and Congressional challenges to certain aspects of PPACA, and we expect there will be additional challenges and amendments in the future. In January, Congress voted to adopt a budget resolution for fiscal year 2017, or the Budget Resolution, that authorizes the implementation of legislation that would repeal portions of PPACA. The Budget Resolution is not a law, however, it is widely viewed as the first step toward the passage of repeal legislation. Further, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of PPACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Congress also could consider subsequent legislation to replace elements of PPACA that are repealed. We cannot predict how PPACA, its possible repeal, or any legislation that may be proposed to replace PPACA will impact our business.

In addition, other legislative changes have been proposed and adopted since PPACA was enacted. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which began in 2013, pursuant to the Budget Control Act of 2011. On March 1, 2013, the President signed an executive order implementing the 2% Medicare payment reductions, and on April 1, 2013, these reductions went into effect. Pursuant to the Bipartisan Budget Act of 2015, these reductions will stay in effect through 2025, unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and, accordingly, our financial operations.

Although we are unsure of the full impact that PPACA will have on our business, we expect that PPACA, 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 receive for any approved drug. Any reduction in reimbursement from Medicare or other government-funded programs may result in a similar reduction in payments from private payors. 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 bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our drugs.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenue, if any.

In some countries, particularly in the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt

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of marketing approval for a drug. To obtain coverage and reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate to other available therapies. If reimbursement of our drugs is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

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 harm 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. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

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

Risks Related to Employee Matters and Managing Our Growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, research and development, clinical, financial and business development expertise of Rachel King, our President and Chief Executive Officer; John Magnani, our Senior Vice President of Research and Chief Scientific Officer; Helen Thackray, our Senior Vice President of Clinical Development and Chief Medical Officer; and Brian Hahn, our Chief Financial Officer, as well as the other members of our scientific and clinical teams. In particular, we are dependent upon Dr. Magnani for key expertise in carbohydrate chemistry and knowledge of carbohydrate biology with respect to our glycomimetics platform, and the loss of his services would materially impair our future drug discovery efforts. Although we have entered into employment agreements with our executive officers, each of them may currently terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or employees other than Ms. King.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our drug pipeline other than rivipansel toward scaling up for commercialization, sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval for and commercialize

our drug candidates. Competition to hire qualified personnel in our industry is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting

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or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

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

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

Our employees and employees of our collaborators may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We and our collaborators are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with FDA regulations, to provide accurate information to the FDA, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations, to report financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended 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, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have 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 improper activities 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. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, or any such actions are instituted against any of our collaborators, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions and diminished royalties.

Risks Related to Ownership of Our Common Stock

An active trading market for our common stock may not continue to develop or be sustained.

Prior to our IPO in January 2014, there was no public market for our common stock. Although our common stock is listed on The Nasdaq Global Market, we cannot assure you that an active trading market for our shares will continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for investors in our common stock to sell shares without depressing the market price for the shares or to sell the shares at all.

The trading price of the shares of our common stock has been and is likely to continue to be volatile.

Since our IPO, our stock price has been volatile. The stock market in general and the market for biopharmaceutical 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, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

· announcements relating to development, regulatory approvals or commercialization of our drug candidates;

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- actual or anticipated variations in our operating results;
- changes in financial estimates by us or by any securities analysts who might cover our stock;
- conditions or trends in our industry;
- changes in laws or other regulatory actions affecting us or our industry;
- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- capital commitments;
- investors' general perception of our company and our business;
- disputes concerning our intellectual property or other proprietary rights;
- recruitment or departure of key personnel; and
- sales of our common stock, including sales by our directors and officers or specific stockholders.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources from our business.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. Even if we have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

The issuance of additional stock in connection with financings, acquisitions, investments, our stock incentive plan, our employee stock purchase plan or otherwise will dilute all other stockholders.

Our certificate of incorporation authorizes us to issue up to 100,000,000 shares of common stock and up to 5,000,000 shares of preferred stock with such rights and preferences as may be determined by our board of directors. Subject to compliance with applicable rules and regulations, we may issue our shares of common stock or securities convertible into our common stock from time to time in connection with a financing, acquisition, investment, our stock incentive plan, our employee stock purchase plan or otherwise. Any such issuance could result in substantial dilution to our existing stockholders and cause the trading price of our common stock to decline.

If a substantial number of our total outstanding shares are sold into the market, or if the market perceives that such sales may occur, it could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or if the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly. All of our outstanding

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shares of common stock are available for sale in the public market, subject only to the restrictions of Rule 144 under the Securities Act in the case of our affiliates.

In addition, we have filed registration statements on Form S-8 registering the issuance of approximately 4.6 million shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under these registration statements are available for sale in the public market subject to vesting arrangements and exercise of options, as well as Rule 144 in the case of our affiliates.

Additionally, some of the holders of our common stock who acquired their shares of our stock prior to the IPO, as well as some of the holders of outstanding warrants to purchase our common stock, or their transferees, have rights, subject to some conditions, to require us to file one or more registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.

There are provisions in our certificate of incorporation and bylaws that may make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change in control was considered favorable by some or all of our stockholders. For example, our board of directors has the authority to issue up to 5,000,000 shares of preferred stock. The board of directors can fix the price, rights, preferences, privileges and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change in control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

Our charter documents also contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors is elected each year;
- stockholders are not entitled to remove directors other than by a 66 2/3% vote and only for cause;
- stockholders are not permitted to take actions by written consent;
- stockholders cannot call a special meeting of stockholders; and
- stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change in control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Our certificate of incorporation also provides that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders.

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 current beneficial owners of 5% or more of our common stock and their respective affiliates beneficially own a majority of our common stock. Further, funds controlled by one investor, New Enterprise Associates, or NEA, beneficially own approximately 26% of our common stock. As a result, NEA is able to significantly influence, and together with these other persons would be able to control, all matters requiring stockholder

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approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets or other significant corporate transactions. The interests of this group of stockholders may not coincide with our interests or the interests of other stockholders.

We are an "emerging growth company," and as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- not being required to hold a non-binding advisory vote on executive compensation or obtain stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will 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. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until the earlier of (1) December 31, 2019, (2) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 billion, (3) the last day of the fiscal year 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 million as of the prior June 30th and (4) any date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Securities Exchange Act of 1934, the Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010 and the rules and regulations of The Nasdaq Global Market. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting and perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting. This requires that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts.

We may in the future discover areas of our internal financial and accounting controls and procedures that need improvement. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control

system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

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If we are unable to maintain proper and effective internal controls in the future, we may not be able to produce timely and accurate financial statements, and we may conclude that our internal controls over financial reporting are not effective. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

We do not anticipate paying any cash dividends on our common stock in the foreseeable future and our stock may not appreciate in value.

We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. There is no guarantee that shares of our common stock will appreciate in value or that the price at which our stockholders have purchased their shares will be able to be maintained.

We will incur increased costs and demands upon management as a result of being a public company.

As a newly public company listed in the United States, we have begun, and will continue, particularly after we cease to be an “emerging growth company,” to incur significant additional legal, accounting and other costs. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and Nasdaq, may increase legal and financial compliance costs and make some activities more time consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies.

We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management’s time and attention from revenue-generating activities to compliance activities. If we do not comply with new laws, regulations and standards, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

ITEM 1B.UNRESOLVED STAFF COMMENTS

None.

ITEM 2.PROPERTIES

Our principal offices occupy approximately 42,000 square feet of leased office space in Rockville, Maryland, pursuant to a lease agreement that expires in October 2023. We believe that our properties are generally in good condition, well maintained, suitable and adequate to carry on our business. We believe our capital resources are sufficient to lease any additional facilities required to meet our expected growth needs.

ITEM 3.LEGAL PROCEEDINGS

From time to time, we are subject to litigation and claims arising in the ordinary course of business. We are not currently a party to any material legal proceedings and we are not aware of any pending or threatened legal proceeding against us that we believe could have a material adverse effect on our business, operating results, cash flows or financial condition.

ITEM 4.MINE SAFETY DISCLOSURES

Not applicable.

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PART II

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

Market Information for Common Stock

Our common stock commenced trading on The Nasdaq Global Market under the symbol "GLYC" on January 10, 2014. Prior to our IPO, there was no public market for our common stock. The following table sets forth, for the periods indicated, the high and low reported sales price of our common stock as reported on the Nasdaq Global Market:

Year Ended December 31, 2017	High	Low
First quarter	$ 6.76	$ 5.34
Second quarter	16.94	3.82
Third quarter	15.41	10.06
Fourth quarter	18.25	10.25

Year Ended December 31, 2016	High	Low
First quarter	$ 6.55	$ 3.70
Second quarter	9.25	5.82
Third quarter	8.89	6.58
Fourth quarter	7.42	5.50

Dividend Policy

We have never declared or paid any dividends on our common stock. We anticipate that we will retain all of our future earnings, if any, for use in the operation and expansion of our business and do not anticipate paying cash dividends in the foreseeable future.

Stockholders

As of February 28, 2018, we had 34,359,799 shares of common stock outstanding held by approximately 32 holders of record. The actual number of stockholders is greater than this number of record holders and includes stockholders who are beneficial owners but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Performance Graph

The following graph compares the performance of our common stock since January 10, 2014, the date of our IPO, with the performance of the Nasdaq Composite Index (U.S.) and the Nasdaq Biotechnology Index. The comparison assumes a $100 investment on January 10, 2014 in our common stock, the stocks comprising the Nasdaq Composite Index, and the stocks comprising the Nasdaq Biotechnology Index, and assumes reinvestment of the full amount of all dividends, if any. Historical stockholder return is not necessarily indicative of the performance to be expected for any future periods.

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Comparison of Cumulative Total Return

Among GlycoMimetics, Inc., the Nasdaq Composite Index and the Nasdaq Biotechnology Index

The performance graph shall not be deemed to be incorporated by reference by means of any general statement incorporating by reference this Form 10-K into any filing under the Securities Act of 1933, as amended or the Exchange Act, except to the extent that we specifically incorporate such information by reference, and shall not otherwise be deemed filed under such acts.

Recent Sales of Unregistered Securities

None.

Purchases of Equity Securities by the Issuer and Affiliated Parties

None.

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ITEM 6.SELECTED FINANCIAL DATA

The following selected financial data as of and for the years ended December 31, 2017, 2016, 2015, 2014 and 2013 is derived from our audited financial statements, which have been audited by Ernst & Young LLP, independent registered public accounting firm. The statement of operations data for the years ended December 31, 2014 and 2013, and the balance sheet data as of December 31, 2015, 2014 and 2013, have been derived from our audited financial statements which are not included herein. Our historical results are not necessarily indicative of the results to be expected in the future. The selected financial data should be read together with Item 7. “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and in conjunction with the financial statements, related notes, and other financial information included elsewhere in this Annual Report.

	Year Ended December 31,				
(in thousands, except share and per share data)	2017	2016	2015	2014	2013
Statement of Operations Data:					
Revenue	$ —	$ 18	$ 20,071	$ 15,027	$ 3,993
Costs and expenses:					
Research and development expense	24,100	23,282	25,050	19,571	11,701
General and administrative expense	9,832	8,650	7,805	6,596	2,899
Total costs and expenses	33,932	31,932	32,855	26,167	14,600
Loss from operations	(33,932)	(31,914)	(12,784)	(11,140)	(10,607)
Other income	651	104	15	18	1
Net loss and comprehensive loss	$ (33,281)	$ (31,810)	$ (12,769)	$ (11,122)	$ (10,606)
Net loss per share of common stock—basic and diluted	$ (1.13)	$ (1.50)	$ (0.67)	$ (0.60)	$ (8.87)
Weighted average common shares outstanding, basic and diluted	29,395,756	21,256,312	19,010,587	18,452,252	1,196,162

	As of December 31,				
(in thousands)	2017	2016	2015	2014	2013
Balance Sheet Data:					
Cash and cash equivalents	$ 123,925	$ 40,042	$ 46,803	$ 55,199	$ 2,311
Total assets	128,583	42,388	48,462	57,264	5,283
Total liabilities	8,882	7,087	7,991	6,461	2,376
Total stockholders’ equity	119,701	35,301	40,472	50,803	2,907

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

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes and other financial information included elsewhere in this Annual Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. You should review Item 1A. "Risk Factors" and "Special Note Regarding Forward-Looking Statements" in this Annual Report for a discussion of important factors that could cause actual results to 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 focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Our proprietary glycomimetics platform is based on our expertise in carbohydrate chemistry and our understanding of the role carbohydrates play in key biological processes. Using this expertise and understanding, we are developing a pipeline of proprietary glycomimetics designed to inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection. We believe this represents an innovative approach to drug discovery to treat a wide range of diseases.

Most human proteins are modified by the addition of complex carbohydrates to the surface of the proteins. The addition of these carbohydrate structures affects the functions of these proteins and their interactions with other molecules. Our initial research and development efforts have focused on drug candidates targeting selectins, which are proteins that serve as adhesion molecules and bind to carbohydrates that are involved in the inflammatory component and progression of a wide range of diseases, including hematologic disorders, cancer and cardiovascular disease. Inhibiting specific carbohydrates from binding to selectins has long been viewed as a potentially attractive approach for therapeutic intervention. The ability to successfully develop drug-like compounds that inhibit binding with selectins, known as selectin antagonists, has been limited by the complexities of carbohydrate chemistry. We believe our expertise in carbohydrate chemistry enables us to design selectin antagonists and other glycomimetics that inhibit the disease-related functions of certain carbohydrates.

We are focusing our initial efforts on drug candidates for rare diseases that we believe will qualify for orphan drug designation. Our first drug candidate, rivipansel, is a pan-selectin antagonist being developed for the treatment of vaso-occlusive crisis, or VOC, a debilitating and painful condition that occurs periodically throughout the life of a person with sickle cell disease. We have entered into an agreement with Pfizer Inc., or Pfizer, for the further development and potential commercialization of rivipansel worldwide. Rivipansel has received fast track designation from the U.S. Food and Drug Administration, or FDA, as well as orphan drug designation from the FDA in the United States and from the European Medicines Agency, or EMA, in the European Union. We believe the clinical progress of rivipansel provides evidence of the significant potential of our lead program and our proprietary glycomimetics platform.

Building on our experience with rivipansel, we are developing a pipeline of other glycomimetic drug candidates. Our second glycomimetic drug candidate, GMI-1271, is a specific E-selectin inhibitor, which we are developing to be used in combination with chemotherapy to treat patients with either acute myeloid leukemia, or AML, or multiple myeloma, or MM, both of which are life-threatening hematologic cancers, and potentially other hematologic cancers as well. We have completed an initial Phase 1 trial in healthy volunteers for GMI-1271 and in May 2017 we completed enrollment in a Phase 1/2 clinical trial in defined populations of patients with AML. In December 2017, at the annual meeting of the American Society of Hematology, or ASH, we presented interim clinical data from this Phase 1/2 clinical trial that showed high remission rates and suggested a favorable safety, pharmacokinetic, or PK, and biomarker profile for GMI-1271.

In March 2018, we announced our design for a randomized, double-blind, placebo-controlled Phase 3 clinical trial to evaluate GMI-1271 in individuals with relapsed/refractory AML, which design is aligned with guidance received from the FDA. Based on consultations with the FDA, the single pivotal trial is planned to enroll 380 adult patients at

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approximately 30 to 40 centers in the United States, Canada, Europe and Australia, with enrollment expected to begin in the third quarter of 2018. The primary efficacy endpoint will be overall survival and, importantly, the FDA has indicated that data on overall survival will not need to be censored for transplant in the primary efficacy analysis, meaning that patients who proceed to transplant will continue to be included as part of the survival analysis. The dosing regimen for our planned Phase 3 trial will be the same as for our completed Phase 2 trial. All patients will be treated with standard chemotherapy of either MEC or FAI (fludarabine, cytarabine and idarubicin), with some of the patients randomized to receive GMI-1271 in addition to chemotherapy. Patients receiving GMI-1271 will be dosed for one day prior to initiation of chemotherapy, twice a day through the chemotherapy regimen, and then for two days after the end of chemotherapy. The dose regimen will be fixed, rather than weight-based, which we believe will simplify administration. We plan to offer multiple cycles of consolidation therapy in both arms of the trial for patients who achieve remission. We believe that multiple cycles of treatment in patients who respond may drive an even deeper response in patients treated with GMI-1271. If this is the case, it could lengthen the duration of remission with potential for additional benefit on survival.

Key secondary endpoints of the Phase 3 trial will include the incidence of severe mucositis and remission rate, which will be assessed in a hierarchical fashion for potential inclusion in the product labeling, if GMI-1271 is approved for marketing by the FDA. We expect preliminary results from this trial to be available by the end of 2020.

We have also initiated a Phase 1 multiple ascending dose-escalation trial of GMI-1271 in defined populations of patients with MM and plan to continue enrollment of the trial in 2018. We anticipate initial topline data in the first quarter of 2019 in this trial.

GMI-1271 received orphan drug designation from the FDA in May 2015 for the treatment of patients with AML. In June 2016, GMI-1271 received fast track designation from the FDA for the treatment of adult patients with relapsed or refractory AML and elderly patients aged 60 years or older with AML. In May 2017, GMI-1271 received breakthrough therapy designation from the FDA for the treatment of adult patients with relapsed or refractory AML. In May 2017, the European Commission, based on a favorable recommendation from the EMA Committee for Orphan Medicinal Products, granted orphan designation for GMI-1271 for the treatment of patients with AML.

In February 2018, we entered into an agreement with the Haemato Oncology Foundation for Adults in the Netherlands, or HOVON, to initiate clinical trial startup activities to evaluate GMI-1271 in adults with newly diagnosed AML but who cannot tolerate intensive chemotherapy, as well as in patients with myelodysplastic syndrome, or MDS, with a high risk of leukemia. The HOVON trial will be the first to evaluate GMI-1271, together with decitabine, in this underserved population of AML and MDS patients, who are not considered by their physicians to be candidates for intensive chemotherapy; these two populations represent a significant potential indication expansion opportunity for GMI-1271. HOVON intends to enroll approximately 140 patients in the clinical trial, including a control arm. Patients will be evaluated after three cycles of therapy, and key efficacy endpoints will include remission rate, disease-free survival and overall survival. The trial is expected to start this year and will be conducted in five countries across Europe.

We are also developing an additional drug candidate, GMI-1359, that simultaneously targets both E-selectin and a chemokine receptor known as CXCR4. Since E-selectin and CXCR4 are both adhesion molecules that keep cancer cells in the bone marrow, we believe that targeting both E-selectin and CXCR4 with a single compound could improve efficacy in the treatment of cancers that affect the bone marrow such as AML and MM, as compared to targeting CXCR4 alone. GMI-1359 is currently being evaluated in a Phase 1 single-dose escalation trial of GMI-1359 in healthy volunteers. In this trial, volunteer participants receive a single injection of GMI-1359, after which they are evaluated for safety, tolerability, PK and pharmacodynamics. The randomized, double-blind, placebo-controlled, escalating dose study is being conducted at a single site in the United States.

In addition to our programs described above, we are also advancing other preclinical-stage programs. These programs include small-molecule glycomimetic compounds that inhibit the protein galectin-3, which we believe may have potential to be used for the treatment of fibrosis, cancer and cardiovascular disease.

We commenced operations in 2003, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, developing our glycomimetics platform, identifying potential drug

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candidates, undertaking preclinical studies and conducting clinical trials of rivipansel, GMI-1271 and GMI-1359. To date, we have financed our operations primarily through private placements of our securities, upfront and milestone payments under our collaboration with Pfizer and the net proceeds from our IPO in January 2014, at-the-market sales facilities with Cowen and Company LLC, or Cowen, and our public offerings of common stock in June 2016 and May 2017. We have no approved drugs currently available for sale, and substantially all of our revenue to date has been revenue from the upfront and milestone payments from Pfizer, although we have received nominal amounts of revenue under research grants.

Prior to our IPO, we raised an aggregate of $86.6 million to fund our operations, of which $22.5 million was an upfront payment under our collaboration with Pfizer and $64.1 million was from the sale of our convertible promissory notes and convertible preferred stock. The IPO provided us with net proceeds of $57.2 million, and we received a non-refundable milestone payment from Pfizer in May 2014 of $15.0 million. In August 2015, we received another non-refundable milestone payment from Pfizer of $20.0 million following the dosing of the first patient in the Phase 3 clinical trial of rivipansel. We received an additional $19.7 million in net proceeds from our public offering in June 2016 and $86.8 million in net proceeds from our public offering in May 2017. During the years ended December 31, 2016 and 2017, we received an aggregate of $30.5 million of net proceeds from sales of our common stock pursuant to our sales agreements with Cowen.

Since inception, we have incurred significant operating losses. We have generated cumulative revenue of $58.6 million since our inception through December 31, 2017, primarily consisting of the $22.5 million upfront payment from Pfizer in 2011, the $15.0 million non-refundable milestone payment in May 2014 and the $20.0 million non-refundable milestone payment in August 2015. We had an accumulated deficit of $152.3 million as of December 31, 2017, and we expect to continue to incur significant expenses and operating losses over at least the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year, depending on the timing of our clinical trials, the receipt of milestone payments, if any, under our collaboration with Pfizer, and our expenditures on other research and development activities. We anticipate that our expenses will increase substantially as we:

- initiate and conduct our planned clinical trials of GMI-1271 and GMI-1359;
- continue the research and development of our other drug candidates;
- seek to discover and develop additional drug candidates;
- seek regulatory approvals for any drug candidates other than rivipansel that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure and scale up external manufacturing capabilities to commercialize any drug candidates other than rivipansel for which we may obtain regulatory approval;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our drug development and potential future commercialization efforts.

To fund further operations, we will need to raise capital. We may obtain additional financing in the future through the issuance of our common stock, through other equity or debt financings or through collaborations or partnerships with other companies. We may not be able to raise additional capital on terms acceptable to us, or at all, and any failure to raise capital as and when needed could compromise our ability to execute on our business plan. Although it is difficult to predict future liquidity requirements, we believe that our existing cash and cash equivalents, together with interest thereon, will be sufficient to fund our operations at least through the end of 2019. However, our ability to successfully transition to profitability will be dependent upon achieving a level of revenues adequate to support our cost structure. We cannot assure you that we will ever be profitable or generate positive cash flow from operating activities.

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Our Collaboration with Pfizer

In October 2011, we entered into the license agreement with Pfizer under which we granted Pfizer an exclusive worldwide license to develop and commercialize products containing rivipansel for all fields and uses. The license also covers specified back-up compounds along with modifications of and improvements to rivipansel that meet defined chemical properties. Pfizer is required to use commercially reasonable efforts, at its expense, to develop, obtain regulatory approval for and commercialize rivipansel for sickle cell disease in the United States. Under the terms of the agreement, we received a $22.5 million upfront payment. We are also eligible to earn potential milestone payments of up to $115.0 million upon the achievement of specified development milestones, including the dosing of the first patients in Phase 3 clinical trials for up to two indications and the first commercial sale of a licensed product in the United States and selected European countries for up to two indications, up to $70.0 million upon the achievement of specified regulatory milestones, including the acceptance of our filings for regulatory approval by regulatory authorities in the United States and Europe for up to two indications, and up to $135.0 million upon the achievement of specified levels of annual net sales of licensed products. We are also eligible to receive tiered royalties for each licensed product, with percentages ranging from the low double digits to the low teens, based on net sales worldwide, subject to reductions in specified circumstances.

The first potential milestone payment under the Pfizer agreement was $35.0 million upon the initiation of dosing of the first patient in a Phase 3 clinical trial of rivipansel by Pfizer. Under the collaboration, Pfizer made a $15.0 million non-refundable milestone payment to us in May 2014, which we recognized as revenue in May 2014, when earned, and the dosing of the first patient in the Phase 3 clinical trial in June 2015 triggered the remaining $20.0 million milestone payment to us. We recorded the $20.0 million milestone payment as revenue in June 2015. There were no milestone payments received from Pfizer for the years ended December 31, 2017 and 2016.

We entered into a research services agreement with the University of Basel, or the University, for the discovery and evaluation of selectin antagonists. The research under this agreement has been completed; however, certain patents covering the rivipansel compound remain subject to provisions of the research services agreement. Under the terms of the Research Agreement, we will owe to the University 10% of all future milestone and royalty payments received from Pfizer with respect to rivipansel. In February 2016, we paid $2.0 million to the University based upon the non-refundable milestone payments from Pfizer. We recorded these payments during the year ended December 31, 2015, at the time the payments became due to the University. There were no additional payments due to the University for the years ended December 31, 2017 and 2016.

Critical Accounting Policies and Significant Judgments and Estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the balance sheets and the reported amounts of revenue and expenses during the reporting periods. In accordance with GAAP, we base our estimates on historical experience and on various other assumptions that we believe are reasonable under the circumstances at the time such estimates are made. Actual results may differ materially from our estimates and judgments under different assumptions or conditions. We periodically review our estimates in light of changes in circumstances, facts and experience. The effects of material revisions in estimates are reflected in our financial statements prospectively from the date of the change in estimate.

We define our critical accounting policies as those accounting principles generally accepted in the United States that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our significant accounting policies are more fully described in Note 2 to our financial statements appearing elsewhere in this Annual Report, we believe the following are the critical accounting policies used in the preparation of our financial statements that require significant estimates and judgments.

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Revenue Recognition

License and Collaboration Agreements

We have entered into a license agreement with Pfizer. Under the agreement, Pfizer made a nonrefundable $22.5 million upfront payment to us in 2011, a $15.0 million milestone payment in 2014 and a $20.0 million milestone payment in 2015. Pfizer may become obligated to make additional milestone payments to us upon the achievement of significant clinical development milestones, regulatory approvals and sales-based events.

The agreement also contemplates royalty payments to us on any future net sales of rivipansel worldwide.

Collaborative research and development agreements can provide for one or more of upfront license fees, research payments and milestone payments. Agreements with multiple components, such as deliverables or similar items, are referred to as multi-element revenue arrangements and are evaluated according to the provisions of Accounting Standards Codification, or ASC, Topic 605-25, Revenue Recognition—Multiple-Element Arrangements, to determine whether the deliverables can be separated into more than one unit of accounting. An item can generally be considered to be a separate unit of accounting if both of the following criteria are met:

- the delivered item(s) has value to our customer on a standalone basis; and
- the arrangement includes a general right of return relative to the delivered item(s), and delivery or performance of the undelivered item(s) is considered probable and substantially in our control.

Items that cannot be divided into separate units are combined with other units of accounting, as appropriate. Consideration received is then allocated among the separate units based on a selling price hierarchy. The selling price hierarchy for each deliverable is based on vendor-specific objective evidence, or VSOE, if it is available; third-party evidence of selling price, or TPE, if VSOE is not available; or an estimated selling price, if neither VSOE nor TPE is available.

Our license agreement with Pfizer represents a multiple-element revenue arrangement. To account for this transaction, we determined the elements, or deliverables, included in the arrangement and allocated arrangement consideration to the various elements based on each element’s relative selling price. The identification of individual elements in a multiple-element arrangement and the estimation of the selling price of each element involve significant judgment, including consideration as to whether each delivered element has standalone value to our collaborator.

The primary deliverable under our license arrangement with Pfizer is an exclusive worldwide license to rivipansel, which is currently being developed to treat people experiencing VOC. The arrangement also includes deliverables related to research and preclinical development activities to be performed by us on Pfizer’s behalf and our participation on a joint steering committee. We concluded that these deliverables should be accounted for as a single unit of accounting, and we therefore determined to recognize the upfront payment of $22.5 million as revenue over the expected development period of 1.5 years, which was the period over which we expected to provide our research and development services and participate on the joint steering committee under the arrangement. Our determination of the appropriate length of the period over which to recognize revenue was consistent with the research plan agreed to with Pfizer and the actual development timeline.

In reaching this conclusion, we evaluated whether the license to rivipansel has standalone value to Pfizer. Factors we considered in determining whether the license has standalone value included whether or not Pfizer can use the license for its intended purpose without the receipt of the remaining deliverables, the value of the license without the undelivered items, Pfizer’s or other vendors’ ability to provide the undelivered items, the proprietary nature of the

license and know-how, and the availability of our glycomimetics expertise in the general marketplace. Based on all relevant facts and circumstances and, most significantly, on the proprietary nature of our platform and the related proprietary nature of our research services, we concluded that standalone value does not exist for the license and, therefore, the license is not a separate unit of accounting under the collaboration and should be combined with the research and development services we are obligated to provide, including our participation on the joint steering committee.

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We also evaluated whether our participation on the joint steering committee is a substantive obligation and therefore a separate unit of accounting. The joint steering committee is responsible for overseeing the general working relationships, determining the protocols to be followed in the research and development performed and evaluating the results from the continued development of the drug candidate. The factors we considered in determining if our participation on the joint steering committee is a substantive obligation included:

- which party negotiated or requested the steering committee;
- how frequently the steering committee meets;
- whether or not there are any penalties or other recourse if we do not attend the steering committee meetings;
- which party has decision-making authority on the steering committee; and
- whether or not Pfizer has the requisite experience and expertise associated with the research and development of rivipansel.

We considered that we may terminate our participation on the joint steering committee at any point during the agreement. Further, the estimated selling price of our obligation was not material to the overall license agreement. Based on all relevant facts and circumstances, we concluded that our participation on the joint steering committee is not a substantive obligation and, therefore, is not a separate unit of accounting under the collaboration.

We were not able to establish VSOE or TPE for the separate unit deliverables under the arrangement with Pfizer, as we do not have a history of entering into such arrangements or selling the individual deliverables within such arrangements separately. Accordingly, we determined that the selling price for the deliverables under the Pfizer license agreement should be determined using the best estimate of selling price. The process of determining the best estimate of selling price involved significant judgment on our part and included consideration of multiple factors, including market conditions and company-specific factors, such as those factors contemplated in negotiating the agreement and internally developed models that included assumptions related to market opportunity, discounted cash flows, estimated development costs, probability of success and the time needed to commercialize a drug candidate pursuant to the license. In validating the best estimate of selling price, we considered whether changes in key assumptions used to determine the best estimate of selling price would have a significant effect on the allocation of the arrangement consideration between the multiple deliverables.

Our license agreement with Pfizer also includes contingent milestone payments related to specified development, regulatory and commercial milestones. Pursuant to ASC-605-28, Revenue Recognition-Milestone Method, we may recognize revenue contingent upon the achievement of a substantive milestone in its entirety in the period the milestone is achieved. Milestones are considered substantive if all of the following conditions are met:

- the milestone is nonrefundable;
- achievement of the milestone was not reasonably assured at the inception of the arrangement;
- substantive effort is involved to achieve the milestone;
- the amount of the milestone appears reasonable in relation to the effort expended or the risk associated with achievement of the milestone; and
- a reasonable amount of time passes between the upfront license payment and the first milestone payment, as well as between each subsequent milestone payment.

Our determination as to whether a payment meets these five conditions involves management’s judgment. If any of these conditions are not met, the resulting payment would not be considered a substantive milestone and would instead be considered part of the consideration for the single unit of accounting. In addition, if we determine that one milestone is not substantive, it could prevent us from concluding that subsequent milestones are substantive and, as a result, any additional milestone payments could also be considered part of the consideration for the single unit of accounting and would be recognized as revenue as those performance obligations are performed under either the proportional performance method or the straight-line method.

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We have evaluated whether each milestone under the Pfizer arrangement is substantive and at risk to both parties on the basis of the contingent nature of that milestone. This evaluation included an assessment of whether:

- the consideration is commensurate with either our performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from our performance to achieve the milestone;
- the consideration relates solely to past performance; and
- the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement.

Based on this evaluation, we concluded that the milestones under the Pfizer collaboration are substantive, due to the uncertainty of future clinical development success and the additional effort and time that is expected before the milestones could be achieved. Accordingly, each milestone will be recognized as revenue upon its achievement, assuming all other revenue recognition criteria are met.

Effective January 1, 2018, we adopted Accounting Standards Codification, or ASC, Topic 606, Revenue from Contracts with Customers, using the full retrospective transition method. Under this method, we will need to revise our financial statements, if necessary, for the years ended December 31, 2015 and 2016, and applicable interim periods within those years, as if Topic 606 had been effective for those periods. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services in an amount that reflects the consideration which the entity expects to receive in exchange for those goods and services. To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with the customer(s); (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue with the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration to which we are entitled in exchange for the goods and services we transfer to the customer. At contract inception, we assess the goods or services promised within each contract that falls under the scope of Topic 606 and determine those that are performance obligations, and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied. We have analyzed the Pfizer agreement and have concluded that the transition to the new revenue standard will have no impact on prior reporting periods.

Stock-Based Compensation

We issue stock-based compensation awards to our employees and non-employee directors, including stock options. We measure stock-based compensation expense related to these awards based on the fair value of the award on the date of grant and recognize stock-based compensation expense on a straight-line basis over the requisite service period of the awards, which generally equals the vesting period. We grant stock options with exercise prices equal to the estimated fair value of our common stock on the date of grant. We have selected the Black-Scholes-Merton option pricing model to determine the fair value of stock option awards, which requires the input of various assumptions that require management to apply judgment and make assumptions and estimates, including:

Risk-Free Interest Rate—The risk-free interest rate assumption is based on observed interest rates for constant maturity U.S. Treasury securities consistent with the expected life of our employee stock options.

Expected Term—The expected life represents the period of time the stock options are expected to be outstanding and is based on the simplified method. Under the simplified method, the expected life of an option is presumed to be the midpoint between the vesting date and the end of the contractual term. We used the simplified method due to the lack of sufficient historical exercise data to provide a reasonable basis upon which to otherwise estimate the expected life of the stock options.

Expected Volatility—Expected volatility is based on the historical volatilities of a peer group of comparable publicly traded companies with drug candidates in similar stages of development along with the Company's historical volatility since its public offering, to determine its expected volatility.

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Expected Dividend Yield—We have assumed no dividend yield because we do not expect to pay dividends in the future, which is consistent with our history of not paying dividends.

Effective on January 1, 2017 with the adoption of ASU 2016-09, we have elected to account for forfeitures as they occur. We applied this change using a modified retrospective method through a cumulative-effect adjustment of $19,727 to accumulated deficit as of January 1, 2017. Previously, the amount of stock-based compensation expense recognized during a period was based on the value of the portion of the awards that were ultimately expected to vest. The estimated pre-vesting forfeitures, or forfeiture rates, were based on our analysis of historical behavior by stock option holders. The estimated forfeiture rate was applied to the total estimated fair value of the awards, as derived from the Black-Scholes-Merton model, to compute the stock-based compensation expense, net of pre-vesting forfeitures, to be recognized in our statements of operations. We estimated forfeitures for employee grants at the time of grant and revised the estimates, if necessary, in subsequent periods if actual forfeitures differed from those estimates.

Research and Development Expenses

Research and development costs are charged to expense as incurred and include employee-related expenses, including salaries, benefits and travel, expenses incurred under agreements with CROs and investigative sites that conduct preclinical studies and clinical trials, as well as the cost of acquiring, developing and manufacturing clinical trial materials, facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies and costs associated with preclinical activities and regulatory operations.

We record costs for some development activities, such as clinical trials, based on our evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided to us by our vendors on their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense, as the case may be.

Income Taxes

On December 22, 2017, the president signed into law the Tax Cuts and Jobs Act (H.R. 1), or the Act. The Act includes a number of changes in existing tax law impacting businesses. One of the most significant changes is a permanent reduction in the corporate income tax rate from 35% to 21%. The rate reduction took effect on January 1, 2018. However, we are required to recognize the effect of this rate change on our deferred tax assets and liabilities in 2017, the year in which the tax rate change was enacted. The net deferred tax asset represents expected corporate tax benefits anticipated to be realized in the future. As a result of the reduction in rate, our deferred tax assets have been revalued with a reduction of $15.2 million, which is offset by the related reduction in our valuation allowance. The SEC staff issued Staff Accounting Bulletin, or SAB, No. 118, which will allow us to record provisional amounts during a measurement period which is similar to the measurement period used when accounting for business combinations. While we have substantially completed our provisional analysis of the income tax effects of this recent tax reform legislation, and recorded a reasonable estimate of such effects, the ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, further refinement of our calculations, additional analysis, changes in assumptions, and actions we may take as a result of the Act.

We recorded deferred tax assets of $56.4 million as of December 31, 2017, which have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits. The deferred tax assets

are primarily composed of federal and state tax net operating loss, or NOL, carryforwards and research and development tax credit carryforwards. As of December 31, 2017, we had federal and state NOL carryforwards of $112.0 million, research and development tax credit carryforwards of $8.0 million, and $13.3 million of orphan drug tax credit carryforwards available to reduce future taxable income, if any. A portion of the net operating loss carryforwards will begin to expire in 2026, the research and development tax credits in 2023 and the orphan drug tax credit in 2033. In general, if we experience a greater than 50 percentage point aggregate change in ownership of specified significant stockholders over a three-year period, utilization of our pre-change NOL carryforwards will be subject to an annual limitation under Section 382 of the U.S. Internal Revenue Code of 1986, as amended, and similar state laws. Such limitations may result in expiration of a portion of the NOL carryforwards before utilization and may be substantial. If we experience a

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Section 382 ownership change as a result of changes in our stock ownership, some of which changes may be outside our control, the tax benefits related to the NOL carryforwards may be further limited or lost.

Components of Operating Results

Revenue

To date, we have not generated any revenue from the sale of our drug candidates and do not expect to generate any revenue from the sale of drugs in the near future. Substantially all of our revenue recognized to date has consisted of the upfront and milestone payments under our agreement with Pfizer.

Since our inception, we have also recognized a nominal amount of revenue under research grant contracts, generally to the extent of our costs incurred in connection with specific research or development activities.

Research and Development

Research and development expenses consist of expenses incurred in performing research and development activities, including compensation and benefits for full-time research and development employees, facilities expenses, overhead expenses, cost of laboratory supplies, clinical trial and related clinical manufacturing expenses, fees paid to CROs and other consultants and other outside expenses. Other preclinical research and platform programs include activities related to exploratory efforts, target validation, lead optimization for our earlier programs and our proprietary glycomimetics platform.

To date, our research and development expenses have related primarily to the development of rivipansel and our other drug candidates. As of April 2013, when we completed our Phase 2 clinical trial of rivipansel, all further clinical development obligations associated with rivipansel have shifted to Pfizer.

We do not currently utilize a formal time allocation system to capture expenses on a project-by-project basis because we are organized and record expense by functional department and our employees may allocate time to more than one development project. Accordingly, we only allocate a portion of our research and development expenses by functional area and by drug candidate.

Research and development costs are expensed as incurred. Non-refundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

Research and development activities are central to our business model. Drug candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later stage clinical trials. We expect our research and development expenses to increase over the next several years as we seek to progress GMI-1271, GMI-1359 and our other drug candidates through clinical development. However, it is difficult to determine with certainty the duration and completion costs of our current or future preclinical studies and clinical trials of our drug candidates, or if, when or to what extent we will generate revenues from the commercialization and sale of any of our drug candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our drug candidates.

The duration, costs and timing of clinical trials and development of our drug candidates will depend on a variety of factors that include:

- per patient trial costs;
- the number of patients that participate in the trials;
- the number of sites included in the trials;
- the countries in which the trial is conducted;
- the length of time required to enroll eligible patients;

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- the number of doses that patients receive;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- the duration of patient follow-up; and
- the safety and efficacy profile of the drug candidate.

In addition, the probability of success for each drug candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each drug candidate, as well as an assessment of each drug candidate's commercial potential.

General and Administrative

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services. We anticipate that our general and administrative expenses will increase in the future to support our continued research and development activities.

Other Income

Other income consists of interest income earned on our cash and cash equivalents.

Results of Operations for the Years Ended December 31, 2017 and 2016

The following table sets forth our results of operations for the years ended December 31, 2017 and 2016.

	YEAR ENDED DECEMBER 31,		PERIOD-TO PERIOD
(in thousands)	2017	2016	CHANGE
Revenue	$ —	$ 18	$ (18)
Costs and expenses:			
Research and development expense	24,100	23,282	818
General and administrative expense	9,832	8,650	1,182
Total costs and expenses	33,932	31,932	2,000
Loss from operations	(33,932)	(31,914)	(2,018)
Other income	651	104	547
Net loss and comprehensive loss	$ (33,281)	$ (31,810)	$ (1,471)

Research and Development Expense

Research and development expense increased by $0.8 million to $24.1 million for the year ended December 31, 2017, from $23.3 million in the year ended December 31, 2016, reflecting an increase of 4%. This increase was primarily due to the manufacturing costs related to clinical supplies for GMI-1271 as we advance towards a planned Phase 3

clinical trial. These increases were offset by a decrease in clinical development expenses as the GMI-1271 Phase 2 clinical trial enrollment was completed in May 2017. In addition, personnel-related and stock-based compensation expenses increased in 2017 by approximately $0.9 million due to an increase in labor-related costs and stock-based compensation expense.

The following table summarizes our research and development expense by functional area for the years ended December 31, 2017 and 2016:

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(in thousands)	YEAR ENDED DECEMBER 31, 2017	2016	PERIOD-TO PERIOD CHANGE
Clinical development	$ 5,700	$ 7,729	$ (2,029)
Manufacturing and formulation	6,625	4,449	2,176
Contract research services, consulting and other costs	1,575	1,998	(423)
Laboratory costs	1,923	1,719	204
Personnel-related	6,996	6,354	642
Stock-based compensation	1,281	1,033	248
Research and development expense	$ 24,100	$ 23,282	$ 818

The following table summarizes our research and development expense by drug candidate for the years ended December 31, 2017 and 2016:

(in thousands)	YEAR ENDED DECEMBER 31, 2017	2016	PERIOD-TO PERIOD CHANGE
GMI-1271	$ 12,488	$ 10,483	$ 2,005
GMI-1359	806	2,875	(2,069)
Other research and development	2,529	2,537	(8)
Personnel-related and stock-based compensation	8,277	7,387	890
Research and development expense	$ 24,100	$ 23,282	$ 818

General and Administrative Expense

The following table discloses the components of our general and administrative expense for the year ended December 31, 2017 and 2016:

(in thousands)	YEAR ENDED DECEMBER 31, 2017	2016	PERIOD-TO PERIOD CHANGE
Personnel-related	$ 3,183	$ 2,829	$ 354
Stock-based compensation	2,480	1,932	548
Legal, consulting and other professional expenses	3,466	3,249	217
Other	703	640	63
General and administrative expense	$ 9,832	$ 8,650	$ 1,182

General and administrative expense increased for the year ended December 31, 2017 by $1.2 million, or 14%, compared to 2016 primarily due to an increase in labor-related costs and stock-based compensation expense.

Results of Operations for the Years Ended December 31, 2016 and 2015

The following table sets forth our results of operations for the years ended December 31, 2016 and 2015.

(in thousands)	YEAR ENDED DECEMBER 31, 2016	2015	PERIOD-TO PERIOD CHANGE
Revenue	$ 18	$ 20,071	$ (20,053)
Costs and expenses:			
Research and development expense	23,282	25,050	(1,768)
General and administrative expense	8,650	7,805	845
Total costs and expenses	31,932	32,855	(923)
Loss from operations	(31,914)	(12,784)	(19,130)
Other income	104	15	89
Net loss and comprehensive loss	$ (31,810)	$ (12,769)	$ (19,041)

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Revenue

Our revenue for the year ended December 31, 2016 was not material. The revenue recorded in the year ended December 31, 2015 was due to the $20.0 million non-refundable milestone payment from Pfizer triggered upon the dosing of the first patient in the Phase 3 clinical trial of rivipansel. There were no milestone or royalty payments due from Pfizer during the year ended December 31, 2016.

Research and Development Expense

Research and development expense decreased by $1.8 million to $23.3 million for the year ended December 31, 2016, from $25.1 million in the year ended December 31, 2015, reflecting a decrease of 7%. This decrease was primarily due to a milestone license fee of $2.0 million payable to the University based on a Pfizer milestone payment received during the year ended December 31, 2015 that did not recur during the year ended December 31, 2016. As part of the original consideration for entering into an agreement with the University, we granted to the University the right to receive 10% of payments related to rivipansel under specified circumstances including any future milestone payments or royalties we receive from Pfizer. The milestone license fee reflected in the year ended December 31, 2015 was based on 10% of the associated non-refundable milestone payments of $20 million due from Pfizer to us. In contrast, there were no milestone or royalties received from Pfizer in year ended December 31, 2016. In addition, during the year ended December 31, 2016, as compared to the same periods in 2015, there was an increase in the costs associated with the clinical development for GMI-1271 and GMI-1359 of $3.6 million offset by a decrease in expenses related to manufacturing and process development for GMI-1271 of $4.3 million.

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The following table summarizes our research and development expense by functional area for the years ended December 31, 2016 and 2015:

(in thousands)	YEAR ENDED DECEMBER 31, 2016	2015	PERIOD-TO PERIOD CHANGE
Clinical development	$ 7,729	$ 4,135	$ 3,594
Manufacturing and formulation	4,449	8,769	(4,320)
Contract research services, consulting and other costs	1,998	2,285	(287)
Laboratory costs	1,719	1,508	211
Personnel-related	6,354	5,549	805
Stock-based compensation	1,033	802	231
Milestone license fee	—	2,002	(2,002)
Research and development expense	$ 23,282	$ 25,050	$ (1,768)

The following table summarizes our research and development expense by drug candidate for the years ended December 31, 2016 and 2015:

(in thousands)	YEAR ENDED DECEMBER 31, 2016	2015	PERIOD-TO PERIOD CHANGE
GMI-1271	$ 10,483	$ 10,770	$ (287)
GMI-1359	2,875	3,256	(381)
Rivipansel	—	2,026	(2,026)
Other research and development	2,537	2,647	(110)
Personnel-related and stock-based compensation	7,387	6,351	1,036
Research and development expense	$ 23,282	$ 25,050	$ (1,768)

General and Administrative Expense

The following table discloses the components of our general and administrative expense for the year ended December 31, 2016 and 2015:

(in thousands)	YEAR ENDED DECEMBER 31, 2016	2015	PERIOD-TO PERIOD CHANGE
Personnel-related	$ 2,829	$ 2,363	$ 466
Stock-based compensation	1,932	1,521	411
Legal, consulting and other professional expenses	3,249	3,334	(85)
Other	640	587	53
General and administrative expense	$ 8,650	$ 7,805	$ 845

General and administrative expense increased for the year ended December 31, 2016 by $845,000, or 11%, compared to 2015 primarily due to an increase in labor-related costs and stock-based compensation expense.

Liquidity and Capital Resources

Sources of Liquidity

We have financed our operations primarily through private placements of our capital stock, our IPO, sales agreements with Cowen, our public offerings in June 2016 and May 2017 and upfront and milestone payments from Pfizer. As of December 31, 2017, we had $123.9 million in cash and cash equivalents.

We are potentially eligible to earn a significant amount of milestone payments and royalties under our agreement with Pfizer. Our ability to earn these payments and their timing is dependent upon the outcome of Pfizer’s activities and is uncertain at this time.

On March 1, 2016, we entered into an at-the-market sales agreement with Cowen to sell shares of our common stock having an aggregate offering price of up to $40.0 million through Cowen acting as our sales agent. During the year ended December 31, 2017, we sold an aggregate of 1,388,647 shares of our common stock under the at-the-market

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facility, for net proceeds of $7.4 million. We and Cowen terminated the agreement in May 2017. As of its termination , we had sold an aggregate of 2,057,438 shares for net proceeds of $11.3 million under the at-the-market facility.

In May 2017, we completed a public offering in which we sold 8,050,000 shares of our common stock at a price to the public of $11.50 per share. We received net proceeds of $86.8 million from this offering, after deducting underwriting discounts, commissions and other offering expenses.

On September 28, 2017, we entered into a new at-the-market sales agreement with Cowen, under which we may offer and sell, from time to time at our sole discretion, shares of our common stock having an aggregate offering price of up to $100.0 million through Cowen acting as our sales agent. As of December 31, 2017, we have sold an aggregate of 1,600,000 shares of our common stock under the new at-the-market facility, for net proceeds of $19.3 million.

Funding Requirements

Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, laboratory and related supplies, clinical costs, legal and other regulatory expenses and general overhead costs.

The successful development of any of our drug candidates is highly uncertain. As such, at this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of GMI-1271 or our other drug candidates. We are also unable to predict when, if ever, material net cash inflows will commence from rivipansel or GMI-1271. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

· successful enrollment in, and completion of, clinical trials;

· receipt of marketing approvals from applicable regulatory authorities;

· establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;

· obtaining and maintaining patent and trade secret protection and regulatory exclusivity for drug candidates;

· launching commercial sales of drugs, if and when approved, whether alone or in collaboration with others; and

· obtaining and maintaining healthcare coverage and adequate reimbursement.

A change in the outcome of any of these variables with respect to the development of any of our drug candidates would significantly change the costs and timing associated with the development of that drug candidate. Because our drug candidates are in various stages of clinical and preclinical development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete the development and commercialization of our drug candidates or whether, or when, we may achieve profitability. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity or debt financings and collaboration arrangements, including our existing collaboration with Pfizer. Except for Pfizer's obligation to make milestone payments under our agreement with them, we do not have any committed external source of liquidity.

To the extent that we raise additional capital through the future sale of equity or debt, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. If we raise additional funds through the issuance of convertible debt securities, these securities could contain covenants that would restrict our operations.

We may require additional capital beyond our currently anticipated amounts. Additional capital may not be available on reasonable terms, or at all. If we raise additional funds through collaboration arrangements in the future, we may have to relinquish valuable rights to our drug 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 when needed, we may be required to delay,

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limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

Outlook

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect that our existing cash and cash equivalents as of December 31, 2017 will enable us to fund our operating expenses and capital expenditure requirements at least through the end of 2019. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we expect. Additionally, the process of testing drug candidates in clinical trials is costly, and the timing of progress in these trials is uncertain.

Cash Flows

The following table summarizes our cash flows for the years ended December 31, 2017, 2016 and 2015.

	YEAR ENDED DECEMBER 31,		
(in thousands)	2017	2016	2015
Net cash provided by (used in):			
Operating activities	$ (29,768)	$ (29,731)	$ (8,242)
Investing activities	(294)	(704)	(269)
Financing activities	113,945	23,674	114
Net change in cash and cash equivalents	$ 83,883	$ (6,761)	$ (8,397)

In assessing cash used in operating activities, we consider several principal factors: (i) net loss for the period; (ii) adjustments for non-cash charges including stock-based compensation expense and depreciation and amortization of property and equipment; and (iii) the extent to which receivables, accounts payable and other liabilities, or other working capital components increase or decrease.

Year Ended December 31, 2017 compared to Year Ended December 31, 2016

Operating Activities

Net cash used in operating activities was $29.8 million during the year ended December 31, 2017 compared to $29.7 million during the year ended December 31, 2016. For the year ended December 31, 2017, there was an increase in cash outlays to reserve manufacturing facilities in 2018 for the production of GMI-1271. These prepaid expenses were offset by a decrease in costs for clinical expenses related to GMI-1271 and GMI-1359.

Investing Activities

Net cash used in investing activities was $294,000 for the year ended December 31, 2017 compared to $704,000 during the year ended December 31, 2016. Net cash used in investing activities for the year ended December 31, 2017 related to the costs associated with purchases of scientific equipment and computers. Net cash used in investing activities for the year ended December 31, 2016 related to the costs associated with the build-out of our additional leased space.

Financing Activities

Net cash provided by financing activities of $113.5 million during the year ended December 31, 2017 reflected net proceeds received from our public offering of $86.8 million in May 2017, $26.7 million received from our at-the-market facilities with Cowen and $0.4 million in proceeds from stock option exercises. Net cash provided by financing activities of $23.7 million during the year ended December 31, 2016 reflected net proceeds received from our public offering of $19.7 million in June 2016 and $3.9 million received from our at-the-market facility with Cowen.

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Year Ended December 31, 2016 compared to Year Ended December 31, 2015

Operating Activities

Net cash used in operating activities was $29.7 million during the year ended December 31, 2016 compared to $8.2 million during the year ended December 31, 2015. For the year ended December 31, 2015, we received a $20.0 million non-refundable milestone payment from Pfizer. In contrast, we did not receive any milestone payments in the year ended December 31, 2016. In addition, there was an increase in expenses of $3.6 million related to clinical development for GMI-1271 and GMI-1359 in 2016 as compared to 2015.

Investing Activities

Net cash used in investing activities was $704,000 for the year end December 31, 2016 compared to $269,000 during the year ended December 31, 2015. Net cash used in investing activities for the year ended December 31, 2016 related to the costs associated with the build-out of our additional leased space. Net cash used in investing activities for the year ended December 31, 2015 was the result of purchases of additional furniture and equipment related to moving into our new office headquarters in June 2015.

Financing Activities

Net cash provided by financing activities of $23.7 million during the year ended December 31, 2016 reflected net proceeds received from our public offering of $19.7 million in June 2016 and $3.9 million received from our at-the-market facility with Cowen. Net cash provided by financing activities of $114,000 during the year ended December 31, 2015 was comprised primarily of employee stock option exercises.

Contractual Obligations

As of December 31, 2017, our significant contractual obligations consisted solely of rent obligations under a non-cancelable lease, as amended, for our current office space in Rockville, Maryland, which has a term through October 2023.

The following table depicts our obligations under this lease as of December 31, 2017.

	Payments Due by Period						
	Total	2018	2019	2020	2021	2022	After 2022
	(In thousands)						
Operating lease	$ 5,981	$ 965	$ 990	$ 1,014	$ 1,040	$ 1,066	$ 906

The foregoing table does not include various agreements that we have entered into for services with third-party vendors, including agreements to conduct clinical trials, to manufacture products, and for consulting and other contracted services due to the cancelable nature of the services. We accrue the costs of these agreements based on estimates of work completed to date.

The contractual obligations table does not include any potential future payments we may be required to make under our research agreement with the University, under which we have agreed to pay 10% of any future milestone payments or royalties we may receive from Pfizer with respect to rivipansel. Due to the uncertainty of the achievement and timing of the events requiring payment under that agreement, the amounts to be paid by us cannot be determined as of the date of this report.

Off-Balance Sheet Arrangements

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

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JOBS Act

In April 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107(b) of the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period, and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

ITEM 7A.QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of December 31, 2017 and 2016, we had cash and cash equivalents of $123.9 million and $40.0 million, respectively. We generally hold our cash in interest-bearing money market accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements and related financial statement schedules required to be filed are listed in the Index to Financial Statements and are incorporated herein.

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

Under the supervision of and with the participation of our management, including our chief executive officer, who is our principal executive officer, and our chief financial officer, who is our principal financial officer, we conducted an evaluation of the effectiveness of our disclosure controls and procedures as of December 31, 2017, the end of the period covered by this Annual Report. The term “disclosure controls and procedures,” as set forth 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 provide reasonable assurance 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 rules and forms promulgated by the SEC. 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 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, 2017,

our chief executive officer and chief financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Report on Internal Control over Financial Reporting and Attestation Report of the Registered Public Accounting Firm

Our management is responsible for establishing and maintaining an adequate system of internal control over financial reporting, as defined in the Exchange Act Rule 13a-15(f). Management conducted an assessment of our internal control over financial reporting based on the original framework established in 2013 by the Committee of Sponsoring

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Organizations of the Treadway Commission in Internal Control—Integrated Framework. Based on the assessment, management concluded that, as of December 31, 2017, our internal control over financial reporting was effective.

This Annual Report does not include an attestation report of our registered public accounting firm regarding internal control over financial reporting as required by Section 404(c) of the Sarbanes Oxley Act of 2002. Because we qualify as an emerging growth company under the JOBS Act, management's report was not subject to attestation by our independent registered public accounting firm.

Changes in Internal Control over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the quarter ended December 31, 2017 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B.OTHER INFORMATION

None.

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PART III

We will file a definitive proxy statement for our 2018 annual meeting of stockholders, or the 2018 Proxy Statement, with the SEC, pursuant to Regulation 14A, not later than 120 days after the end of our fiscal year. Accordingly, certain information required by Part III has been omitted under General Instruction G(3) to Form 10-K. Only those sections of the 2018 Proxy Statement that specifically address the items set forth herein are incorporated by reference.

ITEM 10.DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by Item 10 is hereby incorporated by reference to the relevant information to be included in the 2018 Proxy Statement under the captions “Information Regarding the Board of Directors and Corporate Governance,” “Election of Directors,” “Executive Officers” and “Section 16(a) Beneficial Ownership Reporting Compliance.”

ITEM 11.EXECUTIVE COMPENSATION

The information required by Item 11 is hereby incorporated by reference to the relevant information to be included in the 2018 Proxy Statement under the captions “Executive Compensation” and “Non-Employee Director Compensation.”

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

The information required by Item 12 is hereby incorporated by reference to the relevant information to be included in the 2018 Proxy Statement under the captions “Security Ownership of Certain Beneficial Owners and Management” and “Securities Authorized for Issuance under Equity Compensation Plans.”

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

The information required by Item 13 is hereby incorporated by reference to the relevant information to be included in the 2018 Proxy Statement under the captions “Transactions with Related Persons” and “Independence of the Board of Directors.”

ITEM 14.PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by Item 14 is hereby incorporated by reference to the relevant information to be included in the 2018 Proxy Statement under the caption “Ratification of Selection of Independent Auditors.”

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PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) The following documents are filed as part of this Annual Report on Form 10-K:

(1) Financial Statements:

(2) Financial Statements Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits

Exhibit Number	Description of Document
3.1(1)	Amended and Restated

	Certificate of Incorporation.
3.2(2)	Amended and Restated Bylaws.
4.1(3)	Specimen stock certificate evidencing shares of Common Stock.
10.1*(4)	License Agreement, dated as of October 7, 2011, as amended to date, by and between the Registrant and Pfizer Inc.
10.2(5)	Second Amended and Restated Investor Rights Agreement, dated as of October 20, 2009, by and among the Registrant and certain of its stockholders.
10.3(6)	Form of Common Stock Warrant issued in July 2008 bridge financing.
10.4(7)	Form of Common Stock Warrant issued in

	January 2009 bridge financing.
10.5+(8)	2003 Stock Incentive Plan, as amended.
10.6+(9)	Form of Incentive Stock Option Agreement under 2003 Stock Incentive Plan.
10.7+(10)	Form of Nonqualified Stock Option Agreement under 2003 Stock Incentive Plan.
10.8+(11)	2013 Equity Incentive Plan.
10.9+(12)	Form of Stock Option Grant Notice and Stock Option Agreement under 2013 Equity Incentive Plan.
10.10+(13)	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement under 2013 Equity Incentive Plan.
10.11+(14)	2013 Employee Stock

Purchase Plan.

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Exhibit Number	Description of Document
10.12+(15)	Form of Indemnification Agreement.
10.13+(16)	Amended and Restated Employment Agreement, dated as of January 15, 2014, by and between the Registrant and Rachel King.
10.14+(17)	Amended and Restated Employment Agreement, dated as of January 15, 2014, by and between the Registrant and Brian Hahn.
10.15+(18)	Amended and Restated Employment Agreement, dated as of January 15, 2014, by and between the Registrant and Helen Thackray.
10.16+(19)	Executive Employment Agreement, dated as of May

	19, 2003, by and between the Registrant and John Magnani.
10.17+(20)	Non-Employee Director Compensation Policy.
10.18(21)	Lease Agreement, dated July 23, 2014, by and between the Registrant and BMR-Medical Center Drive, LLC.
10.19(22)	Sales Agreement, dated September 28, 2017 by and between the Registrant and Cowen and Company, LLC.
10.20(23)	First Amendment to Lease, dated March 24, 2016, by and between the Registrant and BMR-Medical Center Drive LLC.
23.1	Consent of Ernst & Young LLP, independent registered public accounting firm.
24.1	Power of Attorney (contained on signature page hereto).

31.1	Certification of Principal Executive Officer pursuant to Rules 13a-14(a) and 15d-14(a) promulgated under 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) and 15d-14(a) promulgated under the Securities Exchange Act of 1934, as adopted pursuant to section 302 of the Sarbanes-Oxley Act of 2002.
32.1^	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Rules 13a-14(b) and 15d-14(b) promulgated under the Securities Exchange Act of

	1934 and 18 U.S.C. Section 1350, as adopted pursuant to section 906 of The Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

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^These certifications are being furnished solely to accompany this Annual Report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

+Indicates management contract or compensatory plan.

*Confidential treatment has been granted with respect to portions of this exhibit (indicated by asterisks) and those portions have been separately filed with the Securities and Exchange Commission.

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(1) Previously filed as Exhibit 3.1 to the Registrant's Current Report on Form 8-K (File No. 001-36177), filed with the Commission on January 15, 2014, and incorporated by reference herein.
(2) Previously filed as Exhibit 3.2 to the Registrant's Current Report on Form 8-K (File No. 001-36177), filed with the Commission on January 15, 2014, and incorporated by reference herein.
(3) Previously filed as Exhibit 4.2 to Amendment No. 2 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 31, 2013, and incorporated by reference herein.
(4) Previously filed as Exhibit 10.1 to Amendment No. 2 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 31, 2013, and incorporated by reference herein.
(5) Previously filed as Exhibit 10.2 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 4, 2013, and incorporated by reference herein.
(6) Previously filed as Exhibit 10.6 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 4, 2013, and incorporated by reference herein.
(7) Previously filed as Exhibit 10.7 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 4, 2013, and incorporated by reference herein.
(8) Previously filed as Exhibit 10.8 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 4, 2013, and incorporated by reference herein.
(9) Previously filed as Exhibit 10.9 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 4, 2013, and incorporated by reference herein.
(10) Previously filed as Exhibit 10.10 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 4, 2013, and incorporated by reference herein.
(11) Previously filed as Exhibit 10.11 to Amendment No. 1 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 28, 2013, and incorporated by reference herein.
(12) Previously filed as Exhibit 10.12 to Amendment No. 1 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 28, 2013, and incorporated by reference herein.
(13) Previously filed as Exhibit 10.13 to Amendment No. 1 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 28, 2013, and incorporated by reference herein.
(14) Previously filed as Exhibit 10.14 to Amendment No. 1 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 28, 2013, and incorporated by reference herein.
(15) Previously filed as Exhibit 10.15 to Amendment No. 1 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on October 28, 2013, and incorporated by reference herein.
(16) Previously filed as Exhibit 10.16 to the Registrant's Annual Report on Form 10-K (File No. 001-36177), filed with the Commission on March 31, 2014, and incorporated by reference herein.
(17) Previously filed as Exhibit 10.17 to the Registrant's Annual Report on Form 10-K (File No. 001-36177), filed with the Commission on March 31, 2014, and incorporated by reference herein.
(18) Previously filed as Exhibit 10.18 to the Registrant's Annual Report on Form 10-K (File No. 001-36177), filed with the Commission on March 31, 2014, and incorporated by reference herein.

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(19) Previously filed as Exhibit 10.19 to the Registrant's Annual Report on Form 10-K (File No. 001-36177), filed with the Commission on March 31, 2014, and incorporated by reference herein.
(20) Previously filed as Exhibit 10.17 to Amendment No. 3 to the Registrant's Registration Statement on Form S-1 (File No. 333-191567), filed with the Commission on December 20, 2013, and incorporated by reference herein.

(21) Previously filed as Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-36177), filed with the Commission on July 28, 2014, and incorporated by reference herein.
(22) Previously filed as Exhibit 1.2 to the Registrant's Registration Statement on Form S-3 (File No. 333-220697), filed with the Commission on September 28, 2017, and incorporated by reference herein.
(23) Previously filed as Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-36177), filed with the Commission on March 29, 2016, and incorporated by reference herein.

ITEM 16. FORM 10-K SUMMARY

Not applicable

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SIGNATURES

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

GLYCOMIMETICS, INC.

By: /s/ Rachel K. King

Rachel K. King
President and Chief Executive Officer

March 6, 2018

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Rachel K. King and Brian M. Hahn, jointly and severally, as his or her true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution, for him or her and in his or her name, place and stead, in any and all capacities, to sign this Annual Report on Form 10-K of GlycoMimetics, Inc., and any or all amendments thereto, 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 full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises hereby ratifying and confirming all that said attorneys-in-fact and agents, or his, her or their substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, 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/ Rachel K. King Rachel K. King	President, Chief Executive Officer and Director (Principal Executive Officer)	March 6, 2018
/s/ Brian M. Hahn	Chief Financial Officer	March 6, 2018

Brian M. Hahn	(Principal Financial Officer and Principal Accounting Officer)	
/s/ Patricia S. Andrews	Director	March 6, 2018
Patricia S. Andrews		
/s/ M. James Barrett, Ph.D.	Director	March 6, 2018
M. James Barrett, Ph.D.		
/s/ Mark A. Goldberg M.D.	Director	March 6, 2018
Mark A. Goldberg M.D.		
/s/ Daniel M. Junius	Director	March 6, 2018
Daniel M. Junius		
/s/ Scott Koenig, M.D., Ph.D.	Director	March 6, 2018
Scott Koenig, M.D., Ph.D.		
/s/ John L. Magnani, Ph.D.	Director	March 6, 2018
John L. Magnani, Ph.D.		
/s/ Timothy Pearson	Director	March 6, 2018
Timothy Pearson		

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Report of Independent Registered Public Accounting Firm

To the Shareholders and the Board of Directors of GlycoMimetics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of GlycoMimetics, Inc. (the Company) as of December 31, 2017 and 2016, the related statements of operations, comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2017 and 2016, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's 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 financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the 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 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 financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company’s auditor since 2011.
Tysons, Virginia
March 6, 2018

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GLYCOMIMETICS, INC.

Balance Sheets

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 123,924,738	$ 40,041,641
Prepaid expenses and other current assets	3,294,884	478,503
Total current assets	127,219,622	40,520,144
Property and equipment, net	1,106,899	1,056,332
Prepaid research and development expenses	204,364	759,531
Deposits	52,320	52,320
Total assets	$ 128,583,205	$ 42,388,327
Liabilities & stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,647,091	$ 1,565,210
Accrued bonuses	1,883,051	1,432,485
Accrued expenses	3,566,607	3,267,371
Deferred rent	78,028	68,551
Total current liabilities	8,174,777	6,333,617
Deferred rent, net of current portion	707,003	753,579
Total liabilities	8,881,780	7,087,196
Stockholders' equity:		
Preferred stock; $0.001 par value; 5,000,000 shares authorized, no shares issued and outstanding at December 31, 2017 and December 31, 2016	—	—
Common stock; $0.001 par value; 100,000,000 shares authorized, 34,359,799 shares issued and outstanding at December 31, 2017; 100,000,000 shares authorized, 23,250,023 shares issued and outstanding at December 31, 2016	34,358	23,249
Additional paid-in capital	271,944,173	154,254,193
Accumulated deficit	(152,277,106)	(118,976,311)
Total stockholders' equity	119,701,425	35,301,131
Total liabilities and stockholders' equity	$ 128,583,205	$ 42,388,327

See accompanying notes.

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GLYCOMIMETICS, INC.

Statements of Operations and Comprehensive Loss

	Year Ended December 31,		
	2017	2016	2015
Revenue	$ —	$ 18,500	$ 20,070,750
Costs and expenses:			
Research and development expense	24,100,092	23,281,820	25,050,179
General and administrative expense	9,832,188	8,650,165	7,805,396
Total costs and expenses	33,932,280	31,931,985	32,855,575
Loss from operations	(33,932,280)	(31,913,485)	(12,784,825)
Other income	651,212	103,647	15,327
Net loss and comprehensive loss	$ (33,281,068)	$ (31,809,838)	$ (12,769,498)
Basic and diluted net loss per common share	$ (1.13)	$ (1.50)	$ (0.67)
Basic and diluted weighted average number of common shares	29,395,756	21,256,312	19,010,587

See accompanying notes.

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GLYCOMIMETICS, INC.

Statements of Stockholders' Equity

	Common Stock Shares	Amount	Additional Paid-In Capital	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2014	18,939,838	$ 18,940	$ 125,181,463	$ (74,396,975)	$ 50,803,428
Exercise of options and warrants	110,366	109	114,012	—	114,121
Stock-based compensation	—	—	2,323,603	—	2,323,603
Net loss	—	—	—	(12,769,498)	(12,769,498)
Balance at December 31, 2015	19,050,204	19,049	127,619,078	(87,166,473)	40,471,654
Issuance of common stock, net of issuance costs	4,145,584	4,146	23,597,809	—	23,601,955
Exercise of options and warrants	54,235	54	72,539	—	72,593
Stock-based compensation	—	—	2,964,767	—	2,964,767
Net loss	—	—	—	(31,809,838)	(31,809,838)
Balance at December 31, 2016	23,250,023	23,249	154,254,193	(118,976,311)	35,301,131
Cumulative effect of adoption of ASU No. 2016-09 for stock-based compensation forfeitures	—	—	19,727	(19,727)	—
Issuance of common stock, net of issuance costs	11,038,647	11,038	113,536,146	—	113,547,184
Exercise of options	71,129	71	373,305	—	373,376
Stock-based compensation	—	—	3,760,802	—	3,760,802
Net loss	—	—	—	(33,281,068)	(33,281,068)
Balance at December 31, 2017	34,359,799	$ 34,358	$ 271,944,173	$ (152,277,106)	$ 119,701,425

See accompanying notes.

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GLYCOMIMETICS, INC.

Statements of Cash Flows

	Year Ended December 31,		
	2017	2016	2015
Operating activities			
Net loss	$ (33,281,068)	$ (31,809,838)	$ (12,769,498)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	263,541	190,540	191,484
Loss on disposal of property and equipment	—	—	8,001
Stock-based compensation expense	3,760,802	2,964,767	2,323,603
Changes in assets and liabilities:			
Prepaid expenses and other current assets	(2,816,381)	(36,693)	474,439
Prepaid research and development expenses	555,167	(63,000)	—
Deposits	—	(52,320)	—
Accounts payable	1,061,880	1,000,975	(309,792)
Accrued expenses and bonuses	724,797	(2,504,179)	1,693,238
Deferred rent	(37,099)	578,483	146,635
Net cash used in operating activities	(29,768,361)	(29,731,265)	(8,241,890)
Investing activities			
Purchases of property and equipment	(294,107)	(704,202)	(268,594)
Net cash used in investing activities	(294,107)	(704,202)	(268,594)
Financing activities			
Proceeds from issuance of common stock, net of issuance costs	113,572,189	23,601,955	—
Proceeds from exercise of stock options	373,376	72,593	114,121
Net cash provided by financing activities	113,945,565	23,674,548	114,121
Net change in cash and cash equivalents	83,883,097	(6,760,919)	(8,396,363)
Cash and cash equivalents, beginning of period	40,041,641	46,802,560	55,198,923
Cash and cash equivalents, end of period	$ 123,924,738	$ 40,041,641	$ 46,802,560
Non-cash investing and financing activities			
Property acquisition costs included in accounts payable and accrued expenses	$ 20,000	$ 21,292	$ —
Issuance costs associated with financing included in accounts payable and accrued expenses	$ 25,005	$ —	$ —

See accompanying notes.

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GLYCOMIMETICS, INC.

Notes to Financial Statements

1. Description of the Business

GlycoMimetics, Inc. (the Company), a Delaware corporation headquartered in Rockville, Maryland, was incorporated on April 4, 2003 and commenced operations on May 21, 2003. The company is a clinical stage biotechnology company focused on the discovery and development of novel glycomimetic drugs to address unmet medical needs resulting from diseases in which carbohydrate biology plays a key role. Glycomimetics are molecules that mimic the structure of carbohydrates involved in important biological processes. Using its expertise in carbohydrate chemistry and knowledge of carbohydrate biology, the Company is developing a pipeline of proprietary glycomimetics that inhibit disease-related functions of carbohydrates, such as the roles they play in inflammation, cancer and infection.

The Company’s executive personnel have devoted substantially all of their time to date to the planning and organization of the Company, the process of hiring scientists, initiating research and development programs and securing adequate capital for anticipated growth and operations. The Company has not commercialized any of its drug candidates and planned commercial operations have not commenced. The Company has incurred significant losses in the development of its drug candidates. The losses in prior periods were primarily attributable to the research and development of the Company’s first drug candidate, rivipansel, as well as GMI-1271 and GMI-1359. The Company has not generated revenues from product sales. As a result, the Company has consistently reported negative cash flows from operating activities and net losses, had an accumulated deficit of $152,277,106 at December 31, 2017 and expects to continue incurring losses for the foreseeable future. The Company currently anticipates that its cash and cash equivalents will be sufficient to meet its anticipated cash requirements through the end of 2019.

The Company’s operations are subject to certain risks and uncertainties. The risks include the need to manage growth, the need to retain key personnel, the need to protect intellectual property, the availability of additional capital financing on terms acceptable to the Company and reliance on its collaboration with Pfizer Inc. (Pfizer). The Company’s current operating assumptions and projections, which reflect management’s best estimate of future revenue and operating expenses, indicate that anticipated operating expenditures through the end of 2019 can be met by available working capital; however, the Company’s ability to meet its projections is subject to uncertainties, and there can be no assurance that the Company’s current projections will be accurate. If the Company’s cash requirements are more than projected, the Company may require additional financing. The type, timing and terms of financing selected by the Company, if required, will be dependent upon the Company’s cash needs, the availability of financing sources and the prevailing conditions in the financial markets. There can be no assurance that such financing will be available to the Company at any given time or available on favorable terms.

Management believes that the Company has access to capital resources through private investments of equity from its existing stockholders. However, it has not secured any commitment for new financing as of the date of this report, nor can it provide any assurance that new financing will be available on commercially acceptable terms, if at all. If the Company is unable to secure additional capital, it will be required to curtail its operations, and if these measures fail, it may not be able to continue its business. Curtailment of operations would cause significant delays in the Company’s efforts to introduce its products to market, which is critical to the realization of its business plan and the future operations of the Company.

2. Summary of Significant Accounting Policies

Basis of Accounting

The accompanying financial statements were prepared based on the accrual method of accounting in accordance with U.S. generally accepted accounting principles (GAAP).

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate

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resources and in assessing performance. The Company views its operations and manages its business in one segment, which is the identification and development of glycomimetic compounds.

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 and disclosure of assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting periods. Although actual results could differ from those estimates, management does not believe that such differences would be material.

Cash and Cash Equivalents

Cash and cash equivalents consist of investment in money market funds with commercial banks and financial institutions. The Company considers all investments in highly liquid financial instruments with an original maturity of three months or less at the date of purchase to be cash equivalents. Cash equivalents are stated at amortized cost, plus accrued interest, which approximates fair value.

Fair Value Measurements

The Company's financial instruments include cash and cash equivalents. The fair values of the financial instruments approximated their carrying values at December 31, 2017 and 2016, due to their short-term maturities. The Company accounts for recurring and nonrecurring fair value measurements in accordance with ASC 820, Fair Value Measurements. ASC 820 defines fair value, establishes a fair value hierarchy for assets and liabilities measured at fair value, and requires expanded disclosures about fair value measurements. The ASC hierarchy ranks the quality of reliability of inputs, or assumptions, used in the determination of fair value, and requires assets and liabilities carried at fair value to be classified and disclosed in one of the following three categories:

- Level 1—Fair value is determined by using unadjusted quoted prices that are available in active markets for identical assets and liabilities.
- Level 2—Fair value is determined by using inputs, other than Level 1 quoted prices, that are directly and indirectly observable. Inputs can include quoted prices for similar assets and liabilities in active markets or quoted prices for identical assets and liabilities in inactive markets. Related inputs can also include those used in valuation or other pricing models that can be corroborated by observable market data.
- Level 3—Fair value is determined by inputs that are unobservable and not corroborated by market data. Use of these inputs involves significant and subjective judgments to be made by a reporting entity. In instances where the determination of the fair value measurement is based on inputs from different levels of fair value hierarchy, the fair value measurement will fall within the lowest level input that is significant to the fair value measurement in its entirety.

The Company periodically evaluates financial assets and liabilities subject to fair value measurements to determine the appropriate level at which to classify them each reporting period. This determination requires the Company to make subjective judgments as to the significance of inputs used in determining fair value and where such inputs lie within the ASC 820 hierarchy.

The Company had no assets or liabilities that were measured using quoted prices for similar assets and liabilities or significant unobservable inputs (Level 2 and Level 3 assets and liabilities, respectively) as of December 31, 2017 and 2016. The carrying value of cash held in money market funds of approximately $121.9 million and $38.0 million as of December 31, 2017 and 2016, respectively, is included in cash and cash equivalents and approximates market values based on quoted market prices (Level 1 inputs).

Concentration of Credit Risk

Credit risk represents the risk that the Company would incur a loss if counterparties failed to perform pursuant to the terms of their agreements. Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. Cash and cash equivalents consist of investment in money market funds

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with major financial institutions in the United States. These deposits and funds may be redeemed upon demand and, therefore, bear minimal risk. The Company does not anticipate any losses on such balances.

Property and Equipment

Property and equipment are recorded at cost and depreciated on a straight-line basis over estimated useful lives ranging from three to seven years. Upon retirement or disposition of assets, the costs and related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in the results of operations. Expenditures for repairs and maintenance are charged to operations as incurred; major replacements that extend the useful life are capitalized. Depreciation and amortization are computed using the straight-line method over the following estimated useful lives:

	ESTIMATED USEFUL LIVES
Furniture and fixtures	7 years
Laboratory equipment	5 years
Office equipment	5 years
Computer equipment	5 years
Computer software	3 years
Leasehold improvements	Shorter of lease term or useful life

Impairment of Long-Lived Assets

The Company periodically assesses the recoverability of the carrying value of its long-lived assets in accordance with the provisions of ASC 360, Property, Plant, and Equipment. ASC 360 requires that long-lived assets and certain identifiable intangible assets be reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. Recoverability of the long-lived asset is measured by a comparison of the carrying amount of the asset to future undiscounted net cash flows expected to be generated by the asset. If the carrying value exceeds the sum of undiscounted cash flows, the Company then determines the fair value of the underlying asset. Any impairment to be recognized is measured by the amount by which the carrying amount of the assets exceeds the estimated fair value of the assets. Assets to be disposed of are reported at the lower of the carrying amount or fair value, less costs to sell. As of December 31, 2017 and 2016, the Company determined that there were no impaired assets and had no assets held for sale.

Revenue Recognition

From time to time, the Company is awarded reimbursement contracts for services and development grant contracts with government and non-government entities and philanthropic organizations. Under these contracts, the Company typically is reimbursed for the costs in connection with specific development activities. The Company recognizes revenue to the extent of costs incurred in connection with performance under such grant arrangements.

The Company has entered into a collaborative research and development agreement with Pfizer. The agreement is in the form of a license agreement. The agreement called for a nonrefundable up-front payment and milestone payments upon achieving significant milestone events. The agreement also contemplates royalty payments on future sales of an approved product. There are no performance, cancellation, termination, or refund provisions in the arrangement that contain material financial consequences to the Company.

The primary deliverable under this arrangement is an exclusive worldwide license to the Company's rivipansel compound, but the arrangement also includes deliverables related to research and preclinical development activities to be performed by the Company on Pfizer's behalf.

Collaborative research and development agreements can provide for one or more of up-front license fees, research payments, and milestone payments. Agreements with multiple components (deliverables or items) are evaluated according to the provisions of ASC 605-25, Revenue Recognition—Multiple-Element Arrangements, to determine whether the deliverables can be separated into more than one unit of accounting. An item can generally be considered a separate unit of accounting if all of the following criteria are met: (1) the delivered item(s) has value to the customer on a stand-alone basis and (2) if the arrangement includes a general right of return relative to the delivered item(s) then delivery or performance of the undelivered item(s) is considered probable and substantially in control of the Company.

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Items that cannot be divided into separate units are combined with other units of accounting, as appropriate. Consideration received is allocated among the separate units based on selling price hierarchy. The selling price hierarchy for each deliverable is based on (i) vendor-specific objective evidence (VSOE), if available; (ii) third-party evidence (TPE) of selling price if VSOE is not available; or (iii) an estimated selling price, if neither VSOE nor third-party evidence is available. Management was not able to establish VSOE or TPE for separate unit deliverables, as the Company does not have a history of entering such arrangements or selling the individual deliverables within such arrangements separately. In addition, there may be significant differentiation in these arrangements, which indicates that comparable third-party pricing may not be available. Management determined that the selling price for the deliverables within the Pfizer collaboration agreement should be determined using its best estimate of selling price. The process of determining the best estimate of selling price involved significant judgment on the Company's part and included consideration of multiple factors such as estimated direct expenses, other costs, and available clinical development data.

Pursuant to ASC 605-25, each required deliverable under the Pfizer collaboration agreement is evaluated to determine whether it qualifies as a separate unit of accounting. Factors considered in this determination include the research capabilities of Pfizer, the proprietary nature of the license and know-how, and the availability of the Company's glycomimetics technology research expertise in the general marketplace. Based on all relevant facts and circumstances and, most significantly, on the proprietary nature of the Company's technology and the related proprietary nature of the Company's research services, management concluded that stand-alone value does not exist for the license, and therefore, the license is not a separate unit of accounting under the contract and will be combined with the research and development services (including participation on a joint steering committee).

Pursuant to ASC 605-28, Revenue Recognition—Milestone Method, at the inception of agreements that include milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether (a) the consideration is commensurate with either (1) the entity's performance to achieve the milestone, or (2) the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the entity's performance to achieve the milestone, (b) the consideration relates solely to past performance and (c) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. In making this assessment, the Company evaluates factors such as scientific, regulatory, commercial and other risks that must be overcome to achieve the respective milestone, the level of effort and investment required to achieve the respective milestone and whether the milestone consideration is reasonable relative to all deliverables and payment terms in the agreement.

Non-refundable development and regulatory milestones that are expected to be achieved as a result of the Company's efforts during the period of substantial involvement are recognized as revenue upon the achievement of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive because the Company does not contribute effort to the achievement of such milestones are generally achieved after the period of substantial involvement and are recognized as revenue upon achievement of the milestone, as there are no undelivered elements remaining and no continuing performance obligation, assuming all other revenue recognition criteria are met. In May 2014, the Company recognized $15.0 million in revenue as a result of the first non-refundable milestone payment received from Pfizer. In June 2015, the Company recognized $20.0 million in revenue as a result of Pfizer dosing the first patient in the Phase 3 clinical trial of rivipansel, which triggered the second non-refundable milestone payment. There were no revenue milestones met in the year ending December 31, 2017 and 2016.

Accrued Liabilities

The Company is required to estimate accrued liabilities as part of the process of preparing its financial statements. The estimation of accrued liabilities involves identifying services that have been performed on the Company's behalf, and then estimating the level of service performed and the associated cost incurred for such services as of each balance sheet date. Accrued liabilities include professional service fees, such as for lawyers and accountants, contract service fees, such as those under contracts with clinical monitors, data management organizations and investigators in conjunction with clinical trials, and fees to contract manufacturers in conjunction with the production of clinical materials. Pursuant to the Company's assessment of the services that have been performed, the Company recognizes these expenses as the services are provided. Such assessments include: (i) an evaluation by the project manager of the work that has been completed during the period; (ii) measurement of progress prepared internally and/or provided by the third-party service provider; (iii) analyses of data that justify the progress; and (iv) the Company's judgment.

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Research and Development Costs

Except for payments made in advance of services, research and development costs are expensed as incurred. For payments made in advance, the Company recognizes research and development expense as the services are rendered. Research and development costs primarily consist of salaries and related expenses for personnel, laboratory supplies and raw materials, sponsored research, depreciation of laboratory facilities and leasehold improvements, and utilities costs related to research space. Other research and development expenses include fees paid to consultants and outside service providers including clinical research organizations and clinical manufacturing organizations.

Stock-Based Compensation

Stock-based payments are accounted for in accordance with the provisions of ASC 718, Compensation—Stock Compensation. The fair value of stock-based payments is estimated, on the date of grant, using the Black-Scholes-Merton model. The resulting fair value is recognized ratably over the requisite service period, which is generally the vesting period of the option.

The Company has elected to use the Black-Scholes-Merton option pricing model to value any options granted. The Company will reconsider use of the Black-Scholes-Merton model if additional information becomes available in the future that indicates another model would be more appropriate or if grants issued in future periods have characteristics that prevent their value from being reasonably estimated using this model.

A discussion of management's methodology for developing some of the assumptions used in the valuation model follows:

Expected Dividend Yield—The Company has never declared or paid dividends and has no plans to do so in the foreseeable future.

Expected Volatility—Volatility is a measure of the amount by which a financial variable such as share price has fluctuated (historical volatility) or is expected to fluctuate (expected volatility) during a period. Prior to the Company's initial public offering, there was not a market for the Company's shares. The Company utilizes the historical volatilities of a peer group (e.g., several public entities of similar size, complexity, and stage of development), along with the Company's historical volatility since its initial public offering to determine its expected volatility.

Risk-Free Interest Rate—This is the U.S. Treasury rate for the week of each option grant during the year, having a term that most closely resembles the expected life of the option.

Expected Term—This is a period of time that the options granted are expected to remain unexercised. Options granted have a maximum term of 10 years. The Company estimates the expected life of the option term to be 6.25 years. The Company uses a simplified method to calculate the average expected term.

Expected Forfeiture Rate—The forfeiture rate is the estimated percentage of options granted that is expected to be forfeited or canceled on an annual basis before becoming fully vested. Effective with the adoption of ASU No. 2016-09 on January 1, 2017, Compensation – Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting, the Company has elected to account for forfeitures as they occur.

Income Taxes

The Company accounts for income taxes using the asset and liability method in accordance with ASC 740, Income Taxes. Deferred income taxes are recognized for the tax consequences in future years of differences between the tax bases of assets and liabilities and the financial reporting amounts at each year-end based on enacted tax laws and statutory tax rates applicable to the periods in which the differences are expected to affect taxable income. A valuation allowance is established when necessary to reduce deferred tax assets to the amount expected to be realized.

The Company accounts for uncertain tax positions pursuant to ASC 740. Financial statement recognition of a tax position taken or expected to be taken in a tax return is determined based on a more-likely-than-not threshold of that tax position being sustained. If the tax position meets this threshold, the benefit to be recognized is measured as the tax

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benefit having the highest likelihood of being realized upon ultimate settlement with the taxing authority. The Company recognizes interest accrued related to unrecognized tax benefits and penalties in the provision for income taxes.

Comprehensive Loss

Comprehensive loss comprises net loss and other changes in equity that are excluded from net loss. For the years ended December 31, 2017, 2016 and 2015, the Company's net loss equals comprehensive loss and, accordingly, no additional disclosure is presented.

Recently Issued Accounting Standards

In March 2016, the FASB issued ASU No. 2016-09, Compensation – Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. This ASU included provisions intended to simplify various aspects related to how share-based payments are accounted for and presented in financial statements including the income tax effects of share-based payments, minimum statutory withholding requirements and forfeitures. The new guidance required all income tax effects of awards to be recognized in the income statement when the awards vest or are settled. It also allows an employer to repurchase more of an employee's shares than the current standard for tax withholding purposes without triggering liability accounting and to make a policy election to account for forfeitures as they occur. The Company adopted the provisions of ASU 2016-09 on January 1, 2017. The Company has elected to account for forfeitures as they occur. The Company has applied this change using a modified retrospective method through a cumulative-effect adjustment of $19,727 to accumulated deficit. Additionally, the Company recognized deferred tax assets of $98,767 for the excess tax benefits that arose directly from tax deductions related to equity compensation greater than the amounts recognized for financial reporting and also recognized an increase of an equal amount in the valuation allowance against those deferred tax assets. The Company has adopted the additional provisions in the standard and has determined these provisions do not have a material impact on the financial statements.

On May 28, 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), requiring an entity to recognize the amount of revenue to which it expects to be entitled for the transfer of promised goods or services to customers. The updated standard replaces most existing revenue recognition guidance in GAAP and permits the use of either the retrospective or cumulative effect transition method. In 2015 and 2016, the FASB issued additional ASUs related to Topic 606 that delayed the effective date of the guidance and clarified various aspects of the new revenue guidance, including principal versus agent considerations, identifying performance obligations, and licensing, and they include other improvements and practical expedients. The Company adopted this new standard on January 1, 2018 using the full retrospective transition method.

Under this method, the Company is required to revise its financial statements, if applicable, for the years ended December 31, 2017 and 2016, and applicable interim periods within those years, as if Topic 606 had been effective for those periods in the year the new standard is adopted. As the Company has concluded that there are no impacts to reported revenue from the adoption of this new standard, no historical amounts will be revised when reporting our year ended December 31, 2018 results or interim periods within that year.

Impact of Adoption

The Company has evaluated the Pfizer Agreement to determine the impact of the new revenue standard on the upfront and milestone payments within the Pfizer Agreement and has determined that the transition to the new revenue standard will have no impact on the financial statements for prior reporting periods. There will be no revised financial line items under Topic 606 for prior year comparative financial statements. For further discussion of the adoption of this standard, see Note 10, “Research and License Agreements”.

Accounting Standards Not Yet Adopted

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842), which supersedes existing guidance on accounting for leases in Leases (Topic 840) and generally requires all leases, including operating leases, to be recognized in the statement of financial position as right-of-use assets and lease liabilities by lessees. The provisions of ASU 2016-02 are to be applied using a modified retrospective approach and are effective for reporting periods beginning after

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December 15, 2018; early adoption is permitted. The Company is currently evaluating the effect that this ASU will have on the financial statements.

With the exception of the new standards discussed above, there have been no new accounting pronouncements that have significance, or potential significance, to the Company's financial statements.

3. Net Loss Per Share of Common Stock

Basic net loss per common share is determined by dividing net loss by the weighted-average number of common shares outstanding during the period, without consideration of common stock equivalents. Diluted net income per share is computed by dividing net income by the weighted-average number of common stock equivalents outstanding for the period. The treasury stock method was used to determine the dilutive effect of the Company's stock option grants, restricted stock units and warrants.

The following table sets forth the computation of basic and diluted earnings per share for the years ended December 31, 2017, 2016 and 2015:

	Year Ended December 31,		
	2017	2016	2015
Net loss	$ (33,281,068)	$ (31,809,838)	$ (12,769,498)
Basic and diluted net loss per common share	$ (1.13)	$ (1.50)	$ (0.67)
Basic and diluted weighted average common shares outstanding	29,395,756	21,256,312	19,010,587

The following potentially dilutive securities outstanding at December 31, 2017, 2016 and 2015 have been excluded from the computation of diluted weighted average shares outstanding, as they would be anti-dilutive:

	Year Ended December 31,		
	2017	2016	2015
Warrants	553,868	553,868	578,687
Stock options and restricted stock units	3,399,124	2,817,674	2,235,775

4. Prepaid Expenses and Other Current Assets

The following is a summary of the Company's prepaid expenses and other current assets at December 31:

	2017	2016
Prepaid research and development expenses	$ 2,941,196	$ 59,004
Other prepaid expenses	251,733	140,191
Other receivables	101,955	268,659
Deposits	—	10,649
Prepaid expenses and other current assets	$ 3,294,884	$ 478,503

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5. Property and Equipment

Property and equipment, net consisted of the following at December 31:

	2017	2016
Furniture and fixtures	$ 314,024	$ 262,135
Laboratory equipment	1,325,667	1,130,180
Office equipment	11,085	6,610
Computer equipment	192,430	169,423
Leasehold improvements	573,165	36,128
Construction in progress	—	508,417
Property and equipment	2,416,371	2,112,893
Less accumulated depreciation	(1,309,472)	(1,056,561)
Property and equipment, net	$ 1,106,899	$ 1,056,332

Depreciation of property and equipment totaled $263,541, $190,540 and $191,484 for the years ended December 31, 2017, 2016 and 2015, respectively.

6. Accrued Expenses

The following is a summary of the Company’s accrued expenses at December 31:

	2017	2016
Accrued research and development expenses	$ 2,702,445	$ 2,513,243
Accrued consulting and other professional fees	227,811	148,579
Other accrued expenses	304,421	315,002
Accrued employee benefits	331,930	290,547
Accrued expenses	$ 3,566,607	$ 3,267,371

7. Operating Leases

The Company leases office and research space in Rockville, Maryland under an operating lease with a term through October 31, 2023 (as amended to date, the Lease) that is subject to annual rent increases. The Company has the right to sublease or assign all or a portion of the premises, subject to the conditions set forth in the Lease. The Lease may be terminated early by either the landlord or the Company in certain circumstances. In connection with the Lease, the Company received rent abatement as a lease incentive. The annual rent increases and rent abatement have been recognized as deferred rent that is being adjusted on a straight-line basis over the term of the Lease.

In March 2016, the Company amended the Lease (the Lease Amendment) to lease additional space beginning on June 1, 2016. In addition to the other terms of the Lease, the Lease Amendment provided for a tenant improvement allowance reflected in the Company's financial statements as an increase in capitalized leasehold improvements as incurred and an increase in deferred rent. In May 2016, the Company also paid a security deposit of $52,320 to be held until the expiration or termination of the Company's obligations under the Lease. The term of the Lease Amendment for the additional space continues through October 31, 2023, the same date as for the premises originally leased under the Lease, subject to the Company's renewal option set forth in the Lease. The Company also has a one-time option to terminate the Lease effective as of October 31, 2020.

Deferred rent related to the Lease was $785,031 and $822,130 at December 31, 2017 and December 31, 2016, respectively. Total rent expense under the Company's leases was $890,170, $784,739 and $595,497 for the years ended December 31, 2017, 2016 and 2015, respectively.

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The following table presents the future minimum lease payments as of December 31, 2017 under the Lease:

YEAR	AMOUNT
2018	$ 965,368
2019	989,502
2020	1,014,239
2021	1,039,595
2022	1,065,585
After 2022	906,669
Total	$ 5,980,958

8. Stockholders' Equity

Common Stock

At-The-Market Equity Offerings

On March 1, 2016, the Company entered into an at-the-market sales agreement with Cowen and Company, LLC to sell the Company's securities under a shelf registration statement filed in March 2015. As of December 31, 2016, the Company had issued and sold 668,791 shares of common stock under the at-the-market sales agreement. The shares were sold at a weighted average price per share of $6.336, for aggregate net proceeds of $3.9 million, after deducting commissions and offering expenses. During the period from January 1, 2017 through May 23, 2017, the Company issued and sold an additional 1,388,647 shares of common stock under the at-the-market sales agreement. The shares were sold at a weighted average price per share of $5.55, for aggregate net proceeds of $7.4 million, after deducting commissions and offering expenses. The at-the market sales agreement was terminated on May 23, 2017.

On September 28, 2017, the Company entered into a new at-the-market sales agreement with Cowen and Company, LLC to sell the Company's securities under a shelf registration statement filed in September 2017. As of December 31, 2017, the Company had issued and sold 1,600,000 shares of common stock under the at-the-market sales agreement. The shares were sold at a weighted average price per share of $12.50, for aggregate net proceeds of $19.3 million, after deducting commissions and offering expenses. As of December 31, 2017, $80.0 million remained available to be sold under the terms of the September 2017 at-the-market sales agreement.

Public Offerings of Common Stock

In June 2016, the Company completed a public offering in which the Company sold 3,476,793 shares of its common stock at a price of $6.10 per share. The Company received net proceeds of $19.7 million from this offering, after

deducting underwriting discounts, commissions and other offering expenses.

In May 2017, the Company completed a public offering in which the Company sold 8,050,000 shares of its common stock at a price to the public of $11.50 per share. The Company received net proceeds of $86.8 million from this offering, after deducting underwriting discounts, commissions and other offering expenses.

Warrants to Acquire Company Stock

The following common stock warrants were outstanding as of each of December 31, 2017 and 2016:

Number of Shares Underlying Outstanding Warrants	Exercise Price Per Share	Expiration Date
236,632	$ 0.33	July 18, 2018
301,986	0.33	January 16, 2019
15,250	0.33	January 30, 2019

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For the year ended December 31, 2017, no warrants were exercised or expired. For the year ended December 31, 2016, a total of 23,275 warrants were exercised at a weighted average exercise price of $0.33 per share. For the year ended December 31, 2015, a total of 11,908 warrants were exercised at a weighted average exercise price of $0.33 per share. For the year ended December 31, 2016, a total of 1,544 warrants expired. No warrants expired during the year ended December 31, 2015.

2003 Stock Incentive Plan

The 2003 Stock Incentive Plan (the 2003 Plan) provided for the grant of incentives and nonqualified stock options and restricted stock awards. The exercise price for incentive stock options must be at least equal to the fair value of the common stock on the grant date. Unless otherwise stated in a stock option agreement, 25% of the shares subject to an option grant will vest upon the first anniversary of the vesting start date and thereafter at the rate of one forty-eighth of the option shares per month as of the first day of each month after the first anniversary. Upon termination of employment by reasons other than death, cause, or disability, any vested options shall terminate 60 days after the termination date. Stock options terminate 10 years from the date of grant. The 2003 Plan expired on May 21, 2013.

A summary of the Company’s stock option activity under the 2003 Plan for the year ended December 31, 2017 is as follows:

	Outstanding Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (In thousands)
Outstanding as of December 31, 2016	729,819	$ 1.26	3.2	
Options exercised	(16,608)	1.65		
Options forfeited	—	—		
Outstanding as of December 31, 2017	713,211	1.25	2.2	$ 11,086
Vested as of December 31, 2017	713,211	1.25	2.2	$ 11,086
Exercisable as of December 31, 2017	713,211	1.25	2.2	$ 11,086

During 2017, 2016 and 2015 the Company issued 16,608, 28,368 and 99,029 shares of common stock, respectively, in conjunction with exercises of stock options granted under the 2003 Plan. The Company received cash proceeds from the exercise of these stock options of approximately to $27,357, $44,771 and $114,124 during 2017, 2016 and 2015, respectively. Total intrinsic value of the options exercised during the years ended December 31, 2017, 2016 and 2015 was $103,638, $97,707 and $667,258, respectively.

As of December 31, 2017, the options under the 2003 Plan were fully expensed. The total fair value of shares vested in the years ended December 31, 2017, 2016 and 2015, was $1,573, $16,024 and $50,310, respectively. There were no options granted from this plan in 2017, 2016 or 2015.

2013 Equity Incentive Plan

The Company’s board of directors adopted, and its stockholders approved, its 2013 Equity Incentive Plan (the 2013 Plan) effective on January 9, 2014. The 2013 Plan provides for the grant of incentive stock options within the meaning of Section 422 of the Internal Revenue Code (the Code), to the Company’s employees and its parent and subsidiary corporations’ employees, and for the grant of nonstatutory stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance stock awards and other forms of stock compensation to its employees, including officers, consultants and directors. The 2013 Plan also provides for the grant of performance cash awards to the Company’s employees, consultants and directors. Unless otherwise stated in a stock option agreement, 25% of the shares subject to an option grant will typically vest upon the first anniversary of the vesting start date and thereafter at the rate of one forty-eighth of the option shares per month as of the first day of each month after the first anniversary. Upon termination of employment by reasons other than death, cause, or disability, any vested options shall terminate 90 days after the termination date, unless otherwise set forth in a stock option agreement. Stock options generally terminate 10 years from the date of grant.

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Authorized Shares

The maximum number of shares of common stock that may be issued under the 2013 Plan was 1,000,000 shares, plus any shares subject to stock options or similar awards granted under the 2003 Plan that expire or terminate without having been exercised in full or are forfeited to or repurchased by the Company. The number of shares of common stock reserved for issuance under the 2013 Plan will automatically increase on January 1 of each year, beginning on January 1, 2015 and ending on January 1, 2023, by 3% of the total number of shares of common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Company's board of directors. The maximum number of shares that may be issued pursuant to exercise of incentive stock options under the 2013 Plan is 20,000,000. As of January 1, 2018, the number of shares of common stock that may be issued under the 2013 Plan was automatically increased by 1,030,793 shares, representing 3% of the total number of shares of common stock outstanding on January 1, 2017, increasing the number of shares of common stock available for issuance under the 2013 Plan to 3,867,994 shares.

Shares issued under the 2013 Plan may be authorized but unissued or reacquired shares of common stock. Shares subject to stock awards granted under the 2013 Plan that expire or terminate without being exercised in full, or that are paid out in cash rather than in shares, will not reduce the number of shares available for issuance under the 2013 Plan. Additionally, shares issued pursuant to stock awards under the 2013 Plan that the Company repurchases or that are forfeited, as well as shares reacquired by the Company as consideration for the exercise or purchase price of a stock award or to satisfy tax withholding obligations related to a stock award, will become available for future grant under the 2013 Plan.

A summary of the Company's stock option activity under the 2013 Plan for the year ended December 31, 2017 is as follows:

	OUTSTANDING OPTIONS	WEIGHTED-AVERAGE EXERCISE PRICE	WEIGHTED-AVERAGE REMAINING CONTRACTUAL TERM(YEARS)	AGGREGATE INTRINSIC VALUE (IN THOUSANDS)
Outstanding as of December 31, 2016	2,066,105	$ 7.41	7.9	
Options granted	674,738	7.03		
Options exercised	(54,521)	6.35		
Options forfeited	(22,159)	7.35		
Outstanding as of December 31, 2017	2,664,163	7.34	7.4	$ 25,091
Vested or expected to vest as of December 31, 2017	2,664,163	7.34	7.4	$ 25,091
Exercisable as of December 31, 2017	1,569,935	7.81	6.5	$ 14,079

The weighted-average fair value of the options granted during the year of December 31, 2017, 2016 and 2015 was $4.76, $3.39 and $5.08 per share, respectively, applying the Black-Scholes-Merton option pricing model utilizing the following weighted-average assumptions:

	2017	2016	2015
Expected term	6.25 years	6.25 years	6.25 years
Expected volatility	75.20%	68.98%	80.76%
Risk-free interest rate	2.08%	1.70%	1.70%
Expected dividend yield	0%	0%	0%

As of December 31, 2017, there was $3,935,996 of total unrecognized compensation expense related to unvested options that will be recognized over a weighted-average period of approximately 2.2 years. The total fair value of shares vested in the years ended December 31, 2017, December 31, 2016 and December 31, 2015 was $3,506,568, $3,053,086 and $2,668,712, respectively. During the years ended December 31, 2017 and December 31, 2016, the Company received cash of $346,019 and $27,825, respectively and issued 54,521 and 3,500 shares of common stock, respectively, in conjunction with exercises of stock options granted under the 2013 Plan. The intrinsic value of the options exercised for the years ended December 31, 2017 and 2016 was $385,701 and $2,275, respectively. There were no option exercises under the 2013 Plan for the year ended December 31, 2015.

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An RSU is a stock award that entitles the holder to receive shares of the Company's common stock as the award vests. The fair value of each RSU is based on the closing price of the Company's stock on the date of grant. The Company has granted RSUs with service conditions (service RSUs) that vest in three equal annual installments provided that the employee remains employed with the Company. As of December 31, 2017, $23,380 of unrecognized compensation costs related to unvested service.

The following is a summary of RSU activity for the 2013 Plan for the year ended December 31, 2017:

	Number of Shares	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2016	16,916	$ 5.04
Granted	—	—
Forfeited	—	—
Vested	7,249	5.61
Unvested at December 31, 2017	9,667	4.61

Stock-based compensation expense was classified as follows on the statement of operations for the years ended December 31:

	2017	2016	2015
Research and development expense	$ 1,280,909	$ 1,033,005	$ 802,329
General and administrative expense	2,479,893	1,931,762	1,521,274
Total stock-based compensation expense	$ 3,760,802	$ 2,964,767	$ 2,323,603

9. Income Taxes

The components of the gross deferred tax asset and related valuation allowance at December 31 were as follows:

	2017	2016
Deferred tax assets:		
Net operating loss carryforward	$ 30,813,509	$ 34,451,814
Capitalized start-up costs	1,695,688	2,708,479
Patent amortization	135,358	216,203
Research and orphan drug credits	21,293,509	14,988,522
Deferred rent	71,938	123,744
Deferred compensation	2,274,885	2,107,964
Other	69,906	70,511
Total gross deferred tax assets	56,354,793	54,667,237
Valuation allowance	(56,354,793)	(54,667,237)
Deferred tax assets	—	—
Deferred tax liabilities:		

Depreciation	—	—
Total deferred tax liabilities	—	—
Net deferred tax assets	$ —	$ —

Based on the Company's operating history and management's expectation regarding future profitability, management believes the realization of the Company's deferred tax assets does not meet the more-likely-than-not criteria under ASC 740, Income Taxes. Accordingly, a full valuation allowance has been established as of December 31, 2017 and 2016.

On December 22, 2017, the Tax Cuts and Jobs Acts (the "TCJA") was enacted into law. The TCJA contains several key tax provisions including the reduction of the corporate income tax rate from 35% to 21% effective January 1, 2018, as well as a variety of other changes, including the limitation of the tax deductibility of interest expense, acceleration of expensing of certain business assets and reductions in the amount of executive pay that can qualify as a

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tax deduction. ASC 740 requires the Company to recognize the effect of the tax law changes in the period of enactment. The Company re-measured certain of its U.S. deferred tax assets and liabilities, based on the rates at which they are expected to reverse in the future. The tax benefit recorded related to the re-measurement of the deferred tax balance was $15.2 million, which was offset by the related valuation allowance. The SEC staff has issued Staff Accounting Bulletin ("SAB") 118, which will allow the Company to record provisional amounts during a measurement period which is similar to the measurement period used when accounting for business combinations. While the company has substantially completed the provisional analysis of the income tax effects of this recent tax reform legislation, and recorded a reasonable estimate of such effects, the ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, further refinement of our calculations, additional analysis, changes in assumptions, and actions we may take as a result of the TCJA.

As of December 31, 2017, the Company had $112.0 million of U.S. Federal and state net operating losses, $8.0 million of research and development tax credits and $13.3 million of orphan drug tax credits available to carry forward. A portion of the net operating loss carryforwards will begin to expire in 2026, the research and development tax credits in 2023 and the orphan drug tax credit in 2033.

The Company's tax attributes, including net operating losses and credits, are subject to any ownership changes as defined under Internal Revenue Code Sections 382 and 383. A change in ownership could affect the Company's ability to utilize its net operating losses and credits. As of December 31, 2017, the Company does not believe that an ownership change has occurred. Any future ownership changes may cause a limitation on the Company's ability to utilize existing tax attributes.

The Company files income tax returns in the U.S. federal jurisdiction and in the State of Maryland. The Company's federal income tax returns for tax years 2003 and after remain subject to examination by the U.S. Internal Revenue Service. The Company's Maryland income tax returns for the tax years 2006 and thereafter remain subject to examination by the Comptroller of Maryland. In addition, all of the net operating losses, research and development tax credit and orphan drug credit carryforwards that may be used in future years are still subject to adjustment.

The Company did not have unrecognized tax benefits as of December 31, 2017 and 2016, and does not anticipate this to change significantly over the next 12 months. The Company will recognize interest and penalties accrued on any unrecognized tax benefits as a component of income tax expense. Reconciliations between the statutory federal income tax rate and the effective income tax rate of income tax expense is as follows as of December 31:

	2017		2016		2015	
U.S. Federal statutory tax rate	34.0	%	34.0	%	34.0	%
State taxes	4.4		4.6		3.7	
Research credit	1.3		1.0		5.6	
Orphan drug credit	11.5		9.0		11.5	
Other	0.3		—		—	
Stock-based compensation	(0.7)		(0.7)		(1.8)	
Change in valuation allowance	(5.1)		(47.9)		(53.0)	
Effective change due to corporate tax rate reduction	(45.7)		—		—	
Provision for income taxes	—	%	—	%	—	%

10. Research and License Agreements

In February 2004, the Company entered into a research services agreement (the Research Agreement) with the University of Basel (the University) for biological evaluation of selectin antagonists. Certain patents covering the rivipansel compound remain subject to provisions of the Research Agreement. Under the terms of the Research Agreement, the Company will owe a 10% payment to the University for all future milestone and royalty payments received from Pfizer with respect to rivipansel. A milestone license fee of $2.0 million was recorded in the year ended December 31, 2015 for the payment due to the University of Basel representing 10% of the $20.0 million non-refundable milestone payment that the Company received from Pfizer in August 2015. The accrued license and milestone fee of $2.0 million was paid in February 2016. There were no payments recorded for the years ended December 31, 2017 or 2016.

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In October 2011, the Company and Pfizer entered into a licensing agreement (the Pfizer Agreement) that provides Pfizer an exclusive worldwide license to rivipansel for vaso-occlusive crisis associated with sickle cell disease and for other diseases for which the drug candidate may be developed. The Company was responsible for completion of the Phase 2 trial, after which Pfizer assumed all further development and commercialization responsibilities. Upon execution of the Pfizer Agreement, the Company received an up-front payment of $22.5 million. The Pfizer Agreement also provides for potential milestone payments of up to $115.0 million upon the achievement of specified development milestones, including the dosing of the first patients in Phase 3 clinical trials for up to two indications and the first commercial sale of a licensed product in the United States and selected European countries for up to two indications; potential milestone payments of up to $70.0 million upon the achievement of specified regulatory milestones, including the acceptance of the Company's filings for regulatory approval by regulatory authorities in the United States and Europe for up to two indications; and potential milestone payments of up to $135.0 million upon the achievement of specified levels of annual net sales of licensed products. Pfizer has the right to terminate the Pfizer Agreement by giving prior written notice.

The Company has determined that each potential future clinical, development and regulatory milestone is substantive. Although sales-based milestones are not considered substantive, they are still recognized upon achievement of the milestone (assuming all other revenue recognition criteria have been met) because there are no undelivered elements that would preclude revenue recognition at that time. The Company is also eligible to receive royalties on future sales contingent upon annual net sales thresholds. In addition, the Company and Pfizer have formed a joint steering committee, or JSC, that will oversee and coordinate activities as set forth in the research program. The $22.5 million up-front payment was recognized over a period of 1.5 years. In May 2014, the Company received a non-refundable payment of $15.0 million from Pfizer as a partial milestone payment owed to the Company upon the dosing of the first patient in the Phase 3 clinical trial. The dosing of the first patient in a Phase 3 clinical trial of rivipansel in June 2015 triggered the remaining $20.0 million of the scheduled milestone payment under the Pfizer Agreement. During the year ended December 31, 2015, the Company recorded revenue of $20.0 million pursuant to the Pfizer Agreement in the Company's statement of operations. There was no revenue recognized under the Pfizer Agreement for the years ended December 31, 2017 and 2016.

The Company assessed this arrangement in accordance with Topic 606 and concluded that the contract counterparty, Pfizer, is a customer. The Company identified the following performance obligations under the contract: (1) an exclusive worldwide license to rivipansel for vaso-occlusive crisis associated with sickle cell disease and for other diseases for which the drug candidate may be developed; and (2) research and development (R&D) services to develop the rivipansel compound for commercial use related to the Phase 2 clinical trial and delivery of data to Pfizer. In addition to the rivipansel license and R&D services, management also considered whether the Company's participation in the JSC constituted a promise. The JSC was formed solely for communication purposes between Pfizer and the Company relating to Pfizer's progress in further developing rivipansel for commercial use. The Company's involvement in the JSC is limited to attending the JSC meetings on a semi-annual basis to receive progress updates from Pfizer; Pfizer is responsible for calling and organizing the meetings. Given the minimal level of involvement by the Company, participation in the JSC is not considered a significant aspect of the arrangement and the related costs, such as employee time, are not material. Therefore, management views the Company's participation in JSC as administrative only and did not further evaluate its participation in the JSC in identifying the performance obligations in the contract.

Under the Agreement, in order to evaluate the appropriate transaction price, the Company determined that the up-front amount constituted the entirety of the consideration to be included in the transaction price and to be allocated to the

performance obligations based on the Company's best estimate of their relative stand-alone selling prices. The transaction price of the upfront fee is equal to the $22.5 million received. The fixed upfront consideration is recognized under ASC 606 based on when control of the combined performance obligation is transferred to the customer, which corresponds with the service period (through March 2013). None of the clinical, regulatory milestones has been included in the transaction price, as all milestone amounts were fully constrained. Event driven milestones are a form of variable consideration as the payments are variable based on the occurrence of future events. As part of its evaluation of the constraint, the Company considered numerous factors, including that receipt of the milestones is outside the control of the Company and contingent upon success in future clinical trials and the licensee's efforts. Recognition of event driven milestones should be recognized when the variable consideration is no longer constrained. There are no changes in accounting necessary for the $15.0 million milestone payment recognized in May 2014 or the $20.0 million milestone payment recognized in June 2015 as a result of Pfizer dosing the first patient in the Phase 3 clinical trial of rivipansel. Future event milestones will be recognized when the constraint no longer applies.

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Any consideration related to sales-based milestones (including royalties) will be recognized when the related sales occur as they were determined to relate predominantly to the license granted to Pfizer and therefore have also been excluded from the transaction price. The Company will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur. In evaluation of the Agreement, there were no significant financing components identified, no non-cash consideration was paid by Pfizer and no consideration was paid by the Company to Pfizer as part of the arrangement.

11. Employee Benefit Plan

The Company has a defined contribution plan under the Internal Revenue Code Section 401(k). This plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. For the years ended December 31, 2017 and 2016, the Company made a discretionary match of 50% up to the first 3% of employee contributions. All matching contributions have been paid by the Company. The Company's matching contributions vest in full at the employee's third anniversary of employment and all employer contributions thereafter vest immediately. The total Company matching contributions were approximately $88,000 and $79,000 for the years ended December 31, 2017 and 2016, respectively. There were no Company matching contributions for the year ended December 31, 2015.

12. Quarterly Financial Information (Unaudited)

Summarized quarterly financial information for each of the years ended December 31, 2017 and 2016 are as follows:

	Quarter Ended December 31, 2017	September 30, 2017	June 30, 2017	March 31, 2017
Revenue	$ —	$ —	$ —	$ —
Net loss	$ (9,257,858)	$ (7,950,101)	$ (8,141,796)	$ (7,931,313)
Loss per share—basic and diluted	$ (0.27)	$ (0.24)	$ (0.30)	$ (0.34)

	Quarter Ended December 31, 2016	September 30, 2016	June 30, 2016	March 31, 2016
Revenue	$ —	$ 18,500	$ —	$ —
Net loss	$ (8,328,597)	$ (7,854,693)	$ (8,071,570)	$ (7,554,978)
Loss per share—basic and diluted	$ (0.36)	$ (0.34)	$ (0.41)	$ (0.40)