MIRAGEN THERAPEUTICS, INC.
Form 10-K
March 14, 2019
UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2018

or

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

For the transition period from to

Commission file number 001-36483

MIRAGEN THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	47-1187261
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

6200 Lookout Road, Boulder, CO 80301
(Address of principal executive offices)

Registrant's telephone number, including area code: (720) 643-5200

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

Title of each class	Name of each exchange on which registered
Common Stock, $0.01 par value	The Nasdaq Capital 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 o No x

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 o No x

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 x No o

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter)
is not contained herein, and will not be contained, to the best of 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. o

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

Large accelerated filer o	Accelerated filer x
Non-accelerated filer o	Smaller reporting company x
Emerging growth company x	

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

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

The aggregate market value of the voting stock held by non-affiliates of the registrant, based upon the closing sale price of the registrant's Common Stock on June 29, 2018, as reported on The Nasdaq Capital Market, was $165.5 million. Shares of Common Stock held by each executive officer and director and by each person who owns 10% or more of the outstanding Common Stock have been excluded in that such persons may be deemed to be affiliates. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 7, 2019, there were 30,921,219 shares of the registrant's Common Stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

None.

MIRAGEN THERAPEUTICS, INC.
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PART I

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, or this Annual Report, contains forward-looking statements that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements contained in this Annual Report, other than statements of historical fact, including statements regarding our strategy, future operations, future financial position, liquidity, future revenue, projected expenses, results of operations, expectations concerning the timing and our ability to report data from ongoing and planned non-clinical studies and clinical trials, prospects, plans and objectives of management are forward-looking statements. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “plan,” “expect,” “predict,” “po “opportunity,” “goals,” or “should,” and similar expressions are intended to identify forward-looking statements. Such statements are based on management’s current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors. Unless otherwise mentioned or unless the context requires otherwise, all references in this Annual Report, to “Miragen,” “company,” “we,” “us” and “our” or similar references refer to Miragen Therapeutics, Inc., and our consolidated subsidiaries.

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

We undertake no obligation to revise or publicly release the results of any revision to these forward-looking statements, except as required by law. Given these risks and uncertainties, readers are cautioned not to place undue reliance on such forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement.

You should also read carefully the factors described in the “Risk Factors” section of this Annual Report on Form 10-K to better understand the risks and uncertainties inherent in our business and underlying any forward-looking statements. You are advised to consult any further disclosures we make on related subjects in our Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and our website.

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ITEM 1. BUSINESS

Merger of Signal Genetics, Inc. and Miragen Therapeutics, Inc.

On February 13, 2017, we, then known as Signal Genetics, Inc., or Signal, completed our merger with Miragen Therapeutics, Inc., a then privately-held Delaware corporation, or Private Miragen. Pursuant to the Agreement and Plan of Merger and Reorganization, or the Merger Agreement, by and among Signal, Private Miragen, and Signal Merger Sub, Inc., a wholly-owned subsidiary of Signal, or Merger Sub, Merger Sub merged with and into Private Miragen, with Private Miragen surviving as a wholly-owned subsidiary of Signal, or the Merger. Immediately, following the Merger, Private Miragen merged with and into us, with us as the surviving corporation, or the Short-Form Merger, and, together with the Merger, the Mergers. In connection with the Short-Form Merger, we changed our corporate name to "Miragen Therapeutics, Inc." Our common stock, par value $0.01 per share, or our common stock, began trading on The Nasdaq Capital Market under the ticker symbol "MGEN" on February 14, 2017.

Overview

We are a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in certain diseases where there is a high unmet medical need. microRNAs are short RNA molecules, or oligonucleotides, that regulate gene expression and play vital roles in influencing the pathways responsible for many disease processes. We have three product candidates, cobomarsen, remlarsen and MRG-110, in clinical development. We believe each has the potential to treat multiple indications. We are independently developing cobomarsen and remlarsen and are developing MRG-110 in collaboration with Les Laboratoires Servier and Institut de Recherches Servier, or, collectively, Servier.

Cobomarsen is an inhibitor of miR-155, which is a microRNA that is found at abnormally high levels in malignant cells of several blood cancers. Remlarsen is a replacement for miR-29, a microRNA that is found at abnormally low levels in a number of pathological fibrotic conditions. MRG-110 is an inhibitor of miR-92, a microRNA expressed in endothelial cells. MRG-110 is being developed for the treatment of heart failure, as well as surgical incisions in high risk populations, severe lacerations, and severe burns. We retain all commercial rights to MRG-110 in the United States and Japan, and Servier has commercial rights to MRG-110 for cardiovascular indications in the rest of the world.

In addition to our clinical-stage programs, we continue to develop a pipeline of preclinical product candidates. The goal of our translational medicine strategy is to progress rapidly to first-in-human trials once we have adequately established the pharmacokinetics (the movement of a drug into, through, and out of the body), pharmacodynamics (the effect and mechanism of action of a drug), safety, and manufacturability of the product candidate in preclinical studies.

We believe our experience in microRNA biology and chemistry, drug discovery, bioinformatics, translational medicine, and drug development allows us to identify and develop mircroRNA-targeted drugs that are designed to regulate gene pathways to return diseased tissues to a healthy state. We believe that our drug discovery and development strategy will enable us to progress our product candidates from preclinical discovery to confirmation of mechanism of action in humans quickly and efficiently. The elements of this strategy include identification of biomarkers that may predict clinical benefit and monitoring outcomes in early-stage clinical trials to help guide later clinical development.

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The following table summarizes our most advanced programs:

_____

(1) Ocular Fibrosis
(2) Idiopathic Pulmonary Fibrosis

Anticipated Milestones

Cobomarsen (blood cancers)
- Phase 1 ATLL clinical trial, additional data release (1H 2019)
- Phase 2 CTCL clinical trial data release (2H 2020)

Remlarsen and other miR-29 mimics (pathologic fibrosis)
- Phase 2 cutaneous fibrosis clinical trial data release (2H 2019)
- Preclinical ocular fibrosis data release (1H 2019)

MRG-110 (heart failure, surgical incisions in high risk populations, severe lacerations, and severe burns)
- Phase 1 systemic clinical trial data release (2019)
- Phase 1 local administration clinical trial data release (2019)

Our Strategy

We seek to use our expertise and understanding of microRNA biology, oligonucleotide chemistry, product development, and manufacturing to create novel products that have the potential to transform the treatment of patients with diseases. The key components of our strategy are as follows:

- Continue to develop cobomarsen for blood cancers. Cobomarsen is currently being developed in two clinical trials for multiple indications. Our global Phase 2 clinical trial, called SOLAR, for cobomarsen in patients with mycosis fungoides, or MF, the most common type of cutaneous T-cell lymphoma, or CTCL, is in the startup phase with initial dosing expected during early 2019. The dosage and administration method of cobomarsen in SOLAR, 300 mg

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intravenous infusion, or IV infusion, demonstrated an objective response in patients that lasted for at least four months (ORR4, the primary outcome in our Phase 2 clinical trial) in 50% of a cohort of eight patients in the Phase 1 clinical trial. In addition to CTCL, we are also developing cobomarsen in three expansion indications where the disease process appears to correlate with an increase in miR-155 levels, the target of cobomarsen. These additional indications are adult T-cell leukemia/lymphoma, or ATLL, diffuse large B-cell lymphoma, or DLBCL, and chronic lymphocytic leukemia, or CLL.

- Continue to develop remlarsen and other miR-29 replacements for pathological fibrosis. In 2018, we initiated a double-blind, randomized Phase 2 clinical trial to evaluate remlarsen in subjects with a predisposition for keloid formation. In 2017, we announced results from the double-blind, placebo-controlled, single and multiple dose-escalation Phase 1 clinical trial evaluating remlarsen in induced cutaneous fibrosis. In the trial, we observed mechanistic proof-of-concept for remlarsen, based on a statistically-significant reduction in scar tissue deposition, with no adverse effects on incisional wound healing when remlarsen was given. Additional indications to be studied for a miR-29 mimic could include fibrotic diseases of the lung and eye.

- Utilize rare disease development pathways at the U.S. Food and Drug Administration, or FDA, and comparable programs at foreign regulatory agencies to accelerate progression to late-stage development and early approval. For our wholly-owned programs, we intend to focus on rare and genetic diseases where RNA modulation may produce clinical benefit, so that we can potentially take advantage of regulatory programs intended to expedite drug development. In 2017, the FDA granted orphan-drug designation to cobomarsen for the treatment of MF and the European Commission granted orphan medicinal product designation to cobomarsen for the treatment of CTCL. We plan to apply for orphan drug designation, fast track, breakthrough therapy designation, and/or priority review when available to potentially reduce clinical trial expense and decrease time to commercialization.

- Collaborate with other biotechnology and pharmaceutical companies to develop additional product candidates. We intend to seek out collaborations for the development of compounds in our pipeline for certain disease areas where the costs would exceed our resources or in other areas where we believe that leveraging a partner's expertise or resources will allow us to accelerate development timelines. For example, we have a strategic collaboration with Servier to develop product candidates for the treatment of cardiovascular diseases.

- Use our in-house research and translational expertise to further develop our product candidate pipeline. Our in-house research team investigates microRNAs that have been identified as potential therapeutic targets through internal efforts and academic collaborations. We then seek to establish evidence that modulation of the microRNAs' activity may provide benefit in pathological conditions or diseases in which the microRNA is implicated. We believe that this internal research and expertise could provide a foundation to develop product candidates for the treatment of a variety of diseases.

- Selectively build focused commercial capabilities and establish commercial collaborations to maximize the value of our product candidate pipeline. To date, we have retained all U.S. and Japanese rights to our product candidates in the strategic collaboration with Servier and global rights in all of our other programs. While we have not yet defined our sales, marketing, or product distribution strategy for cobomarsen, remlarsen, MRG-110, or any of our other product candidates, if approved, our commercial strategy may include the use of strategic alliances, distributors, a contract sales force, or the establishment of our own commercial and specialty sales force to maximize the value of our pipeline.

Our Product Candidates

Cobomarsen

Cobomarsen is an inhibitor of miR-155. Data reported in the scientific literature identifies miR-155 as a cancer-causing microRNA, or oncomiR. There are several types of cancer in which high levels of miR-155 have been observed, including, among others, CTCL, certain virally-induced lymphomas such as ATLL caused by the human T-lymphotropic virus type 1, or HTLV-1, subsets of DLBCL, CLL, and other types of cancer. Based on this literature, miR-155 is implicated in the expression of a number of validated cancer-related disease targets, including Bruton's tyrosine kinase, or BTK, and nuclear factor kappa-light-chain-enhancer of activated B-cells, or NF- B. In certain B-cell lymphomas, improvement of clinical outcomes has been associated with normalization of miR-155 levels, while poor prognosis, resistance to treatment, and recurrence of the disease are associated with elevated levels of miR-155. In addition to playing a role in B-cell malignancies, miR-155 is elevated in another group of malignant white blood cells, called T-cells, found in skin lesions of patients with MF. We screened a library of locked nucleic acid, or LNA, modified oligonucleotides and identified cobomarsen as having what we believed was the best

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potential efficacy and drug-like properties, including improved pharmacodynamics in human T-cell and B-cell lymphoma cell lines. We retain worldwide rights for cobomarsen.

Mycosis Fungoides

MF is a slow growing form of cancer that has been associated with elevated miR-155. This disease is the most common form of a type of blood cancer known as CTCL, which occurs when certain types of T-cells become cancerous. These malignant T-cells then form specific types of skin lesions. Although the skin is involved, the skin cells themselves are not cancerous. According to the National Institutes of Health, or NIH, MF usually occurs in adults over age 50, although the disease may occur at any age.

We believe the total population of patients with CTCL in the United States and Canada is approximately 30,000. The Lymphoma Research Foundation estimated the prevalence of MF to be 16,000-20,000 cases in the United States, with 3,000 new diagnoses of MF each year. According to the Leukemia and Lymphoma Society, or LLS, in a 2014 publication, approximately 70% to 80% of patients are diagnosed with early-stage MF that impacts only the skin. In these patients, the disease typically has a slow progression, but is accompanied by serious quality of life detriments such as severe itchiness, pain, and disfiguration. The five-year survival rate for newly diagnosed patients with CTCL is approximately 90%. As CTCL progresses, the cancer may involve the lymph nodes, blood, and internal organs. The five-year survival rate in later stage patients with CTCL (stages IIB, III, IV) is approximately 20-60% depending on the stage.

There are currently no curative therapies for CTCL, and concurrent and consecutive treatments, many with significant adverse effects, tend to be given until loss of response. We believe there is a need for new and improved therapies in CTCL to treat the disease and reduce symptoms, such as itchiness and painful skin lesions, and to prolong survival in patients with aggressive disease.

There is no universally accepted standard of care, or SOC, for treatment of MF. Treatment is dependent on stage of disease and responsiveness to previous therapy and is divided into skin-directed therapy and systemic treatments. For certain patients with advanced disease, allogeneic stem cell transplantation may offer prolonged survival, but the five-year survival rate is approximately 50%.

In our Phase 1 clinical trial of cobomarsen treating MF patients, we enrolled patients with mild/moderate to severe MF (stages I-III), without blood or node involvement. Cohorts were dosed by multiple routes of administration, including subcutaneous injection, or SQ injection, IV infusion, and intravenous bolus injection, or IV bolus. Efficacy and tolerability were assessed at doses of 300 mg, 600 mg and 900 mg for SQ injection and IV infusion and at 300 mg for IV bolus. Based on the modified Severity Weighted Assessment Tool, or mSWAT score, which is a measurement of the severity of skin disease over a patient’s entire body, 33 of 36 patients (92%) showed improvements in mSWAT scores. These improvements in mSWAT scores were observed as early as 17 days after a patient’s first dose (the first post-treatment assessment), with the greatest improvement in mSWAT scores seen after one or more months of dosing. Additionally, five of eight patients (63%) receiving 300 mg IV infusion have achieved a 50% or greater mSWAT score reduction and four (50%) maintained the response for at least four consecutive months.

Based on results of the Phase 1 clinical trial and the outcomes of FDA meetings during 2018, we advanced cobomarsen into the global Phase 2 SOLAR clinical trial in patients with MF. SOLAR includes an open-label, parallel group, randomized design to evaluate the safety and efficacy of 300 mg of cobomarsen, given by IV infusion, versus Zolinza (vorinostat) as an active control. We have opened a number of clinical sites in the Phase 2 clinical trial, and we are planning to initiate activities at up to 60 clinical sites in 11 countries worldwide. The SOLAR trial is designed to enroll patients with moderate to severe MF (stages Ib-III), without node or blood involvement. The primary endpoint of the SOLAR trial is the rate of objective response that is durable for four months, defined as 50% or

greater improvement in the severity of a patient's skin disease over the entire body (mSWAT), with no evidence of disease progression in the blood, lymph nodes or viscera. Secondary endpoints include progression-free survival and patient reported outcomes measuring overall quality of life as determined by validated instruments. Anticipated enrollment will include approximately 65 patients per treatment group. We expect to report data from SOLAR in the second half of 2020. Based on discussions with the FDA, we believe that a successful outcome for the primary endpoint of the SOLAR trial may allow us to apply for accelerated approval. LLS is collaborating with us and providing funding in support of the SOLAR trial for the treatment of patients with CTCL.

Adult T-cell leukemia/lymphoma

ATLL is a blood cell malignancy that develops in patients after prolonged infection with HTLV-1. Literature suggests that the infection with HTLV-1 as well as the subsequent malignancies may be associated with elevation in the expression of miR-155, the target of cobomarsen. ATLL is typified by abnormalities in blood counts and frequent opportunistic infections. The disease

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presents in multiple forms, but the most common and lethal include the acute, or leukemic, form and the lymphomatous version. These two manifestations lack good treatment options, and once the diagnosis is made, average life expectancy with standard chemotherapeutic treatment regimens is approximately four months for the acute leukemic form and approximately 10 months for the lymphomatous variety.

As of December 13, 2018, eight ATLL patients have been treated with cobomarsen in our expanded Phase 1 clinical trial. Five subjects with acute and lymphomatous ATLL in partial remission have the disease which has remained stable or improved while continuing on cobomarsen monotherapy from three to 13 months. Treatment is ongoing in all subjects at the time of the December 13, 2018 cut-off date for our most recent population. Three subjects, one relapsing with lymphomatous and two relapsing with significant skin involvement, were treated for less than one month and withdrew from the study. The lymphomatous patient relapsing after approximately seven months of chemotherapy was treated with cobomarsen for nine days (four doses). Though lactate dehydrogenase, or LDH, decreased by approximately 50% (3600 U/L on C1D1 to 1554 U/L on C1D12), CT scans showed a mixed response with some nodes decreasing, but others increasing in size, and the patient was discontinued. In addition to patients with clinical stabilization, certain patients have demonstrated a decrease in markers on the circulating tumor cells indicative of disease severity including Ki67, a marker of cell proliferation. Elevations of Ki67 have been shown to correlate with poor life expectancy for ATLL patients. To date, while on cobomarsen monotherapy, there have been no serious adverse effects, or SAEs, reported in patients related to study drug, and none of the patients demonstrated evidence of opportunistic infections.

Diffuse large B-cell lymphoma

According to the Lymphoma Research Foundation, DLBCL is the most common type of non-Hodgkin lymphoma, or NHL, in the United States and worldwide, accounting for up to one-third of patients with newly diagnosed NHL in the United States. DLBCL is a fast-growing form of NHL that affects B-lymphocytes. Lymphocytes are one type of white blood cell. B-cells are lymphocytes that make antibodies to fight infections and are an important part of the immune system. DLBCL can develop in the lymph nodes or in areas outside the lymph nodes, such as the gastrointestinal tract, testes, thyroid, or essentially any organ of the body. It may be in one spot or spread throughout the body.

Approximately 40% of all DLBCL patients have refractory disease or disease that will relapse after an initial response, and the majority of patients with relapsed DLBCL will succumb to the disease. There are two major biologically distinct molecular subtypes of DLBCL: germinal center B-cell and activated B-cell, or ABC. ABC DLBCL is associated with substantially worse outcomes when treated with standard chemoimmunotherapy. One molecular distinction between the two subtypes is the understanding that NF- B, a prosurvival and antiapoptotic molecule, is constitutively expressed and may be a key contributor to chemotherapy resistance in the ABC subgroup. In Part A of our Phase 1 clinical trial, we observed that cobomarsen inhibited the NF- B pathway.

As of January 8, 2019, three DLBCL patients of the ABC subtype had received cobomarsen in the expanded Phase 1 clinical trial. One patient has seen a complete reduction in one of two measured lymph nodes and significant reduction and stabilization in the second lymph node after seven weeks of therapy. As of March 14, 2019, this patient remained on cobomarsen. Two of the three patients discontinued therapy after less than one month due to lack of immediate response. Previously, all three patients had relapsed after multiple cycles of other treatments with other therapies received over 12 to 56 months from diagnosis. Prior treatments for these patients ranged from standard of care to experimental chemotherapy.

We are also currently evaluating cobomarsen in a Phase 1 clinical trial in CLL. We retain worldwide rights for cobomarsen.

Remlarsen

Remlarsen is a replacement for, or mimic of, miR-29. We initially discovered the role of miR-29 in pathological cardiac fibrosis. Since this initial discovery, miR-29 has been implicated in pathological fibrosis in multiple organs including the skin, eye, lung, liver, tendon, muscle, and kidney. miR-29 is understood by the scientific community to play a role in the regulation of certain processes that contribute to fibrosis, including the initiation and maintenance of fibrosis through transforming growth factor beta, or TGF-ß, signaling and the deposition of the components that make up fibrotic tissue, including collagen and extracellular matrix, or ECM, proteins. Furthermore, both fibrotic ECM and TGF-ß are believed to down-regulate miR-29 levels, leading to continuously increased TGF-ß expression and uncontrolled ECM production. miR-29 levels are abnormally low in multiple fibrotic indications, and lower levels of miR-29 are correlated with increased severity of fibrosis. As such, we believe that normalizing levels of miR-29 could be beneficial in the treatment of several pathological fibrotic conditions. Although various fibrotic indications are potentially distinct, they share a number of features, including the activation of the cells that initiate the deposition of fibrotic tissue or fibroblast activation, excessive deposition of collagen and other fibrosis-associated pathways, and resulting organ dysfunction. We believe the functions and biomarkers regulated by miR-29 might be

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shared among multiple fibrotic indications and that increasing miR-29-like activity may provide potential benefit in any of these.

To demonstrate mechanistic proof-of-concept and as a potential initial indication, we initially focused on skin fibrosis. However, we believe data derived from skin fibrosis trials may facilitate development of a product candidate intended for the treatment of patients who suffer from Idiopathic Pulmonary Fibrosis, or IPF, ocular fibrosis, tendon fibrosis, and other major organ pathological fibrosis. We retain worldwide rights for remlarsen.

Pathological Fibrosis

Fibrosis describes the development of fibrous connective tissue as a response to injury or damage. Fibrosis may refer to the deposition of connective tissue that occurs as part of normal healing or to the excess tissue deposition that occurs as a disease process. When fibrosis occurs in response to injury, the term “scarring” is used. Pathological fibrosis can occur in many tissues of the body, either as a primary event or as a result of inflammation or damage. In every case, regardless of the trigger, collagen build up occurs, which can result in scarring of vital organs such as the skin, lung, liver, eye, kidney, tendon, muscle, and heart, leading to irreparable damage and eventual organ failure. In addition, fibrosis prevents the normal healing of the organs and further perpetuates the fibrotic process. We believe there is a significant need for additional clinical therapeutic approaches to treating pathological fibrosis.

Below is a description of several types of pathological fibrosis for which we may seek to develop a product candidate based on a replacement for miR-29:

Type of Pathological Fibrosis	Description
Skin Fibrosis	Scarring is a result of an over production of collagen in a healing wound. Scarring may continue to thicken for up to six months or may overgrow the site of the wound, even after the wound has healed. Hypertrophic scars and keloids are abnormal wound responses and represent an excessive connective tissue response to skin trauma, inflammation, surgery, or burns. Hypertrophic scars and keloids are characterized by local fibroblast proliferation and overproduction of collagen. Both hypertrophic scars and keloids are diseases that tend to be painful and itchy, restrict mobility, and are resistant to treatment.
Pulmonary Fibrosis	Pulmonary fibrosis, also known as lung fibrosis, is caused by accumulation of scar tissues surrounding the air sacs (interstitial space) in the lung. As a result, the lung tissue becomes stiff and loses the ability to expand. The scar tissue also prevents normal transport of oxygen. The result is a progressive respiratory failure, with symptoms that include persistent cough, chest pain, difficulty breathing and fatigue. Pulmonary fibrosis leads to cardiac failure and death. Pulmonary fibrosis may occur as a secondary condition in various other diseases, but in many cases the underlying cause is not clear and is referred to as IPF. IPF is a chronic, progressive lung disease which ultimately leads to death in many of the patients. This condition causes scar tissue to build up in the lungs, which makes the lungs unable to transport oxygen into the bloodstream effectively.
Liver Fibrosis	Liver fibrosis refers to the scar tissue and nodules that replace liver tissue and disrupt liver function. Major causes of liver fibrosis are alcohol, chronic hepatitis B virus infection, hepatitis C virus infection, along with the metabolic disorders non-alcoholic fatty liver disease and non-alcoholic steatohepatitis. Liver fibrosis is a major global problem driven by increasing rates of obesity and diabetes.
Eye Fibrosis	Infection or inflammation of the eye results in impairment of visual function. Chronic inflammation can ultimately lead to fibrosis.

Eye fibrosis diseases include retinal fibrosis such as diabetic retinopathy and proliferative vitreoretinopathy, corneal fibrosis, glaucoma trabeculectomy, age-related macular degeneration, and Fuch's endothelial corneal dystrophy.

Remlarsen Clinical Development

In 2017, we announced the data from a single-center, Phase 1, double-blind, placebo-controlled, single and multiple dose-escalation clinical trial for remlarsen that enrolled 54 healthy volunteers. In the trial, we observed mechanistic proof-of-concept for remlarsen, based on a statistically-significant reduction in fibroplasia, or scar tissue deposition, with no adverse effects on incisional wound healing when remlarsen was given.

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In July 2018, we announced the initiation of a Phase 2 double-blind, randomized clinical trial of remlarsen, which is designed to treat fibrotic diseases, in subjects with a predisposition for keloid formation. This clinical trial is designed to assess the safety, tolerability, and activity of remlarsen in the prevention or reduction of keloid formation in subjects with a history of keloid scars, a persistent form of hypertrophic scarring. Keloids are a common condition that is disfiguring and can be painful, itchy, and emotionally troubling to those that experience them. They are typically smooth, hard, benign growths that form when scar tissue grows excessively. We anticipate that this clinical trial will initially enroll up to 12 patients who are historically predisposed to keloid formation after trauma to the skin at multiple clinical sites in the United States. Subjects will receive small, matching excisional wounds that will be sutured and then injected with either remlarsen or placebo. In this design, patients are serving as their own control, which increases the statistical power of the clinical trial. The lesions will be observed for up to 12 months to determine the presence or absence of keloid formation. We expect to report data from this clinical trial in the second half of 2019. We believe the results of the trial may help determine the dose, dose frequency, and number of patients necessary for a potential Phase 3 clinical trial of remlarsen in keloid revision or other syndromes associated with dermal scarring.

In October 2018, we announced data from our preclinical studies investigating the antifibrotic effects of remlarsen in corneal ulceration of rats. We believe the results obtained in the study suggest that topical application of remlarsen may be an effective treatment to improve vision in patients suffering from multiple conditions resulting in corneal scarring, which remains one of the leading causes of blindness worldwide. We expect to report additional data from these preclinical studies in the first half of 2019.

MRG-110

We are developing MRG-110 (or S95010 per Servier) in collaboration with Servier. MRG-110 is an inhibitor of miR-92, which has been observed in preclinical studies to be a regulator of new blood vessel creation. We believe that miR-92 inhibitors may improve heart function in patients suffering from chronic heart failure, or CHF. We also believe that MRG-110's ability to induce new blood vessel growth may accelerate and improve healing and thus potentially decrease complications of surgically created or naturally occurring wounds in a variety of settings. MRG-110 is our third product candidate to enter human clinical trials and is currently in two Phase 1 clinical trials in collaboration with Servier to evaluate its potential use in the treatment of heart failure and other conditions where patients may benefit from increased vascular flow and accelerated healing, such as complicated lacerations in high risk patients and burns.

Chronic Heart Failure Physiopathology

The imbalance between oxygen demand and supply to cardiomyocytes, or cells in the heart responsible for pumping blood, plays an important role in the pathophysiology of heart failure. CHF is associated with a decrease of myocardial blood flow that begins at the early stages of the heart failure. The preservation of the small blood vessels of the heart is able to increase blood flow in case of increased demand. In CHF, this coronary flow reserve was shown to be reduced secondary to capillary dysfunction and a decrease in density of these vessels, limiting oxygen supply to cardiomyocytes. Analysis of heart tissue from patients suffering from CHF revealed a reduction of coronary microvascular density. Sixty percent of cases of CHF patients with reduced ejection fraction, or HFrEF, have an ischemic origin. Progressive loss of cardiomyocytes and increase in fibrosis decrease capillary density. Compensatory elongation and hypertrophy of remaining cardiomyocytes further increase capillary length and inter-capillary distance reducing oxygenation.

CHF is one of the leading causes of mortality and morbidity in the world. The prognosis remains poor with 45-60% mortality five years after diagnosis. Quality of life in patients is impaired, from mild to severe limitations in daily life. To date, SOC treatment slows down the progression of disease by inhibiting the neuro-hormonal activation and

reducing vascular bed congestion. Coronary revascularization, with percutaneous coronary intervention or coronary arterial bypass grafting, have been shown to improve patient prognosis when the obstruction is located in the epicardial coronaries but is generally of no benefit in cases when the flow is limited downstream in the microcirculatory network. We believe that new reparative/regenerative solutions are needed for improving patient cardiac function that could consequently make a difference in daily quality of life with a further reduction in morbidity and mortality. The restoration of the microcirculation appears to be a potentially innovative therapeutic way to improve cardiac function.

MRG-110 was observed to reduce infarct size in multiple preclinical models of acute myocardial infarction, leading to an improved cardiac function. The cardioprotective effects were correlated with reduced cell death, reduced inflammation, and improved neovascularization of the affected myocardium. Similar effects were also observed in pig hibernating myocardium, a model of chronic ischemia, thought to be more representative of human cardiomyopathies.

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Cutaneous wounds

In preclinical studies, we recently observed MRG-110 accelerating wound healing in normal, healthy farm pigs. In induced excisional wounds in healthy pigs, MRG-110 appeared to result in increased perfusion, measured by laser Doppler imaging on Day 14, and more rapid wound closure compared to wounds in control animals treated similarly with vehicle control or SOC. Within the dermal portion of the wound bed, there was a dose dependent increase in granulation tissue and in vascularization on day 49, five weeks after the last dose, in the wounds treated with MRG-110 compared with SOC-treated wounds. We believe the effects on wound healing seen in animal models support further evaluation of MRG-110 for its potential to improve wound healing by increasing revascularization and granulation tissue formation, and ultimately wound closure in acute settings. The potential indications include acceleration and improvement of wound healing in indications such as burns, skin flaps, grafts, or laparotomy or sternotomy incisions in patients with high risk of poor wound closure.

In 2018, we began evaluating MRG-110 in two Phase 1 clinical trials. Both clinical trials are designed to evaluate the safety, tolerability, and pharmacokinetics of MRG-110. The data generated in these clinical trials is expected to provide several clinically translatable biomarkers that may support future clinical trials. We expect to report data from these clinical trials in 2019.

We are developing MRG-110 with Servier under our license and collaboration agreement, or the Servier Collaboration Agreement. Under the Servier Collaboration Agreement, we granted Servier exclusive licenses to commercialize MRG-110 and other product candidates targeting miR-92 in the field of cardiovascular disease in all countries except the United States and Japan. We also granted Servier the right to name one additional microRNA target under our collaboration agreement through September 2019. We retain all rights to licensed programs under the Servier Collaboration Agreement in the field of cardiovascular disease in the United States and Japan and for all non-cardiovascular indications worldwide.

Background on microRNAs

microRNAs are transcribed from the genome and unlike messenger RNA, or mRNA, they do not encode proteins. microRNAs function by preventing the translation of mRNAs into proteins and/or by triggering degradation of these mRNAs. Studies have shown that microRNA gene regulation is often not a decisive on and off switch but a subtle function that fine-tunes cellular phenotypes that becomes more pronounced during stress or disease conditions. microRNAs were first discovered in 1993 and have since been found in nearly every biological system examined since that time. They are highly conserved across species, demonstrating their importance to biological functions and cellular processes. According to the Sanger Institute, over 2,000 microRNAs have been identified in humans.

A body of evidence has shown that inappropriate levels of particular microRNAs are directly linked to a range of serious diseases, many of which are poorly served by existing therapies. microRNAs can affect the balance of protein expression and serve as “command and control” nodes that directly coordinate multiple critical systems simultaneously. This effect on systems biology is a naturally occurring homeostatic process that becomes disrupted in certain disease states. As a result, developing microRNA-based therapeutics is fundamentally different from the single-protein, single-target approach that is the foundation of traditional small and large molecule drugs.

Our Approach to Drug Discovery and Development

Our research and development strategy is designed to accelerate timelines and reduce development risk. The goal of our translational medicine strategy is to progress rapidly to first-in-human trials once we have adequately established mechanistic proof-of-concept, consisting of pharmacokinetics, pharmacodynamics, safety, and manufacturability of the product candidate in preclinical studies. Programs that progress into human trials are designed to be accompanied

by a validated set of pharmacodynamic biomarkers that allow us to verify the mechanism of drug action in humans and to potentially stratify and enrich the study population. Through this approach, we seek to reduce the risk of our programs by quantifying target engagement and identifying the likely efficacious dose prior to progression to Phase 2 clinical trials.

Discovery

Although there are over 2,000 identified human microRNAs, not all of them have been shown to be causal in disease. Our approach to drug discovery and development begins with the identification of potentially pathological microRNAs.

We apply three general approaches to the identification of potentially pathological, or disease-causing, microRNAs: (i) profiling of microRNA expression in diseased tissue versus normal tissue to identify microRNAs that are found at abnormally high or low levels; (ii) identification of microRNAs that are located within genes (typically in non-protein coding segments) of

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validated disease-relevant genes and thus simultaneously expressed with the disease associated gene; and (iii) evaluation of microRNAs that are predicted to directly modulate the expression of specific, disease-relevant genes.

We believe that the microRNA inhibitor candidates face lower delivery hurdles compared to microRNA mimics and have better drug-like properties in regard to affinity to their targets, stability, drug distribution, and pharmacodynamics. To improve their therapeutic potential, we chemically modify these compounds with changes such as LNA substitution of the ribose sugar in many of the nucleosides, and deoxyribonucleoside, or DNA.

In conditions where a deficit in microRNA expression has been identified as disease causing, microRNA replacements, which are modified, double-stranded RNA structures that are recognized by the RNA-induced silencing complex can serve as chemically-synthesized replacements for microRNAs.

Historically, the delivery of double stranded RNAs, such as microRNA replacements, has been a significant hurdle to overcome for drug development because these molecules are very rapidly degraded and because uptake into cells can be inefficient. Our delivery approach for double-stranded microRNA replacements is to append a conjugate to the molecule to enhance cellular uptake. The selection of the conjugate is dependent upon the intended therapeutic use. We have deployed hydrophobic conjugates, such as cholesterol, that are able to improve pharmacokinetics and allow for enhanced cellular uptake. We are also exploring a range of conjugates that help in targeting specific tissues and cells. Our strategy with microRNA replacements has centered on opportunities for efficient delivery of the molecules with an emphasis on local and topical applications, such as injections in the skin, eye, or lung. For organs where topical or local applications are not feasible, such as the liver, we have employed conjugates that have demonstrated successful delivery after systemic administration.

Development

Our approach to translational medicine is focused on rapidly testing the molecular hypothesis in human cell lines and animal models to demonstrate safety and measure pharmacokinetics and pharmacodynamics, and finally designing and conducting small, efficient, and targeted human Phase 1 clinical trials. We typically select an initial indication that is genetically defined or is a rare disease where abnormal levels of a microRNA have been implicated. These early-stage Phase 1 clinical trials are designed to test the mechanistic relevance and develop mechanistic proof-of-concept in humans in a setting that provides the opportunity to develop a biomarker toolkit for a mechanism of action that we believe has broader disease relevance.

The mechanistic proof-of-concept studies are designed to provide relevant information that helps to reduce development risks in humans. Our aim is to demonstrate that the expression levels of the microRNA could potentially serve as a diagnostic indicator that allows for better patient selection for later clinical trials and in additional indications. At the same time, we seek to confirm molecular activity of the drug.

By measuring the pharmacodynamics of target engagement, we are able to show that the product candidate effectively enters the appropriate cell and binds to its intended target. This process is particularly important for oligonucleotide drugs. We can also measure the effects on a series of downstream genes that create a plausible link between target engagement and a mechanism of disease.

Exploratory endpoints can provide us with verification of the pharmacodynamic effects of the drug based on biomarker readouts and morphological alterations. This translational strategy allows us to answer many questions about the drug target pair and provides improved confidence that the molecular basis of drug action is relevant in humans. Having built confidence in the drug mechanism and demonstrated an acceptable safety profile, later-stage clinical trials will be designed to establish appropriate dose and therapeutic efficacy.

Our Strategic Collaborations and License Agreements

Strategic Alliance and Collaboration with Servier

In October 2011, we entered into the Servier Collaboration Agreement with Servier for the research, development, and commercialization of RNA-targeting therapeutics in cardiovascular disease. Under the Servier Collaboration Agreement, we granted Servier an exclusive license to research, develop, manufacture, and commercialize RNA-targeting therapeutics for certain microRNA targets in the cardiovascular field. In 2017, the Servier Collaboration Agreement was amended to remove all existing targets, add one new target (miR-92), and grant Servier the right to add one additional target through September 2019.

In April 2018, together with Servier, we entered into a seventh amendment to the Servier Collaboration Agreement, or the Servier Amendment. The Servier Amendment, among other things, (i) updated the development plan for MRG-110 and cost-

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sharing provisions; (ii) provided for specified development cost reimbursement by Servier to us following a determination by a joint committee established by the parties under the Servier Collaboration Agreement that the outcome of a specified portion of a Phase 1 clinical trial has met its primary end point; and (iii) provided for additional development plan cost reimbursement by Servier to us following a determination by a joint committee established by the parties under the Servier Collaboration Agreement that a product candidate targeting miR-92 will proceed into a Phase 2 clinical trial.

Servier's rights to each named target are limited to therapeutics in the field of cardiovascular disease, as defined, and in their territory, which is worldwide except for the United States and Japan. We retain all other rights including commercialization of therapeutics developed under the Servier Collaboration Agreement in the field of cardiovascular disease in the United States and Japan.

During 2018, we recorded a development milestone of €3.0 million ($3.7 million). We are eligible to receive non-refundable development milestone payments of €5.8 million to €13.8 million ($6.6 million to $15.8 million as of December 31, 2018) and regulatory milestone payments of €10.0 million to €40.0 million ($11.4 million to $45.8 million as of December 31, 2018) for each target. Additionally, we may receive up to €175.0 million ($200.3 million as of December 31, 2018) in commercialization milestones, as well as quarterly royalty payments expressed in percentages ranging from the low-double digits to the mid-teens (subject to reductions for patent expiration, generic competition, third-party royalty, and costs of goods) on the net sales of any licensed product commercialized by Servier. Servier is obligated to make royalty payments for a period specified under the Servier Collaboration Agreement.

The development of each product candidate (commencing with registration enabling toxicology studies) under the Servier Collaboration Agreement is performed pursuant to a mutually agreed upon development plan to be conducted by the parties as necessary to generate data useful for both parties to obtain regulatory approval of such product candidates. Servier is responsible for a specified percentage of the cost of research and development activities under the development plan through the completion of one or more Phase 2 clinical trials and will reimburse us for a specified portion of such costs that we incur. The costs of Phase 3 clinical trials for each product candidate will be allocated between the parties at a specified percentage of costs. The applicable percentage for each product candidate will be based upon whether certain events under the Servier Collaboration Agreement occur, including if we enter into a third-party agreement for the development and/or commercialization of a product in the United States at least 180 days before the initiation of the first Phase 3 clinical trial, or if we subsequently enter into a U.S. partner agreement, or if we do not enter into a U.S. partner agreement but file for approval in the United States using data from the Phase 3 clinical trial.

During the years ended December 31, 2018 and 2017, we recognized as revenue amounts reimbursable to us under the Servier Collaboration Agreement of $3.7 million and $3.1 million, respectively.

Under the Servier Collaboration Agreement, we also granted Servier a royalty-free, non-exclusive license to develop a companion diagnostic in its territory for any therapeutic product that may be developed by Servier under the Servier Collaboration Agreement. We also granted Servier an exclusive, royalty-free license to commercialize such a companion diagnostic in our territory for use in connection with the development and commercialization of such therapeutic product in its territory.

The Servier Collaboration Agreement will expire as to each underlying product candidate when Servier's royalty obligations as to such product candidate have expired. Servier may also terminate the Servier Collaboration Agreement for: (i) convenience upon a specified number of days' prior notice to us or (ii) upon determination of a safety issue relating to development under the agreement upon a specified number of days' prior notice to us. Either party may terminate the Servier Collaboration Agreement upon a material breach by the other party that is not cured within a specified number of days. We may also terminate the agreement if Servier challenges any of the patents

licensed by us to Servier.

License Agreements with the University of Texas

As of December 31, 2018, we had two exclusive patent license agreements, or the UT License Agreements, with the Board of Regents of The University of Texas System, or the University of Texas. Under each of the UT License Agreements, the University of Texas granted us exclusive and nonexclusive licenses to certain patent and technology rights. The University of Texas is one of our minority stockholders.

In consideration of rights granted by the University of Texas, we agreed to: (i) pay a nonrefundable upfront license documentation fee in the amount of $10 thousand per license; (ii) pay an annual license maintenance fee in the amount of $10 thousand per license starting one year from the date of each agreement; (iii) reimburse the University of Texas for actual costs incurred in conjunction with the filing, prosecution, enforcement, and maintenance of patent rights prior to the effective date;

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and (iv) bear all future costs of and manage the filing, prosecution, enforcement, and maintenance of patent rights. During the years ended December 31, 2018 and 2017, we incurred immaterial upfront and maintenance fees, which were recorded as research and development expense. All costs related to the filing, prosecution, and maintenance of patent and technology rights are recorded as general and administrative expense when incurred.

Under the terms of the UT License Agreements, we may be obligated to make the following future milestone payments for each licensed product candidate: (i) up to approximately $0.6 million upon the initiation of defined clinical trials; (ii) $2.0 million upon regulatory approval in the United States; and (iii) $0.5 million per region upon regulatory approval in other specified regions. Additionally, if we or any of our sublicensees successfully commercialize any product candidate subject to the UT License Agreements, we are responsible for royalty payments in the low-single digits based upon net sales of such licensed products and payments at a percentage in the mid-teens of any sublicense income, subject to specified exceptions. The University of Texas's right to the royalty payments will expire as to each license agreement upon the expiration of the last patent claim subject to the applicable UT License Agreement. During the year ended December 31, 2018, we made an immaterial milestone payment, which is included in research and development expense in our consolidated statements of operations and comprehensive loss. Prior to December 31, 2018, we did not incur any milestone payments.

The license term extends on a product-by-product and country-by-country basis until the expiration of the last to expire of the licensed patents that covers such product in such country. Upon expiration of the royalty payment obligation, we will have a fully paid license in such country. We may also terminate each UT License Agreement for convenience upon a specified number of days' prior notice to the University of Texas. The University of Texas also has the right to earlier terminate the UT License Agreements after a defined date under specified circumstances where we have effectively abandoned our research and development efforts or have no sales. The UT License Agreements will terminate under customary termination provisions including automatic termination upon our bankruptcy or insolvency, upon notice of an uncured material breach, and upon mutual written consent. All charges incurred under the UT License Agreements have been expensed to date due to the uncertainty as to future economic benefit from the acquired rights.

License Agreement with Roche Innovation Center Copenhagen A/S (formerly Santaris Pharma A/S)

In June 2010, we entered into a license agreement with the Santaris Pharma A/S, which was subsequently acquired by F. Hoffmann-La Roche Ltd, or Roche, in 2014, and subsequently changed its name to Roche Innovation Center Copenhagen A/S, or RICC. The agreement was amended in October 2011 and amended and restated in December 2012, or the RICC License Agreement.

Under the RICC License Agreement, we received exclusive and nonexclusive licenses from RICC to use specified technology of RICC, or the RICC Technology, for specified uses including research, development, and commercialization of pharmaceutical products using this technology worldwide. Under the RICC License Agreement, we have the right to develop and commercialize the RICC Technology directed to four specified targets and the option to obtain exclusive product licenses for up to six additional targets. The acquisition of Santaris Pharma A/S by Roche was considered a change-of-control under the RICC License Agreement, and as such, certain terms and conditions of the RICC License Agreement changed, as contemplated and in accordance with the RICC License Agreement. These changes primarily relate to milestone payments reflected in the disclosures below. If we exercise our option to obtain additional product licenses or to replace the target families, we will be required to make additional payments to RICC.

Under the terms of the RICC License Agreement, milestone payments were previously decreased by a specified percentage as a result of the change of control by RICC referenced above. We are obligated to make milestone payments for each licensed product of up to $5.2 million, which is inclusive of a potential product license option fee. Certain of these milestones will be increased by a specified percentage if we undergo a change-of-control during the

term of the RICC License Agreement. If we grant a third party a sublicense to the RICC Technology, we are required to remit to Roche up to a specified percentage of the upfront and milestone and other specified payments that we receive under its sublicense, and if such sublicense covers use of the RICC Technology in the United States or the entire European Union, or EU, we will not have any further obligation to pay the fixed milestone payments noted above.

If we or our sublicensee successfully commercializes any product candidate subject to the RICC License Agreements, then RICC is entitled to royalty payments in the mid-single digits on the net sales of such product, provided that if such net sales are made by a sublicensee under the RICC License Agreement, RICC is entitled to royalty payments equal to the lesser of a percentage in the mid-single digits on the net sales of such product or a specified percentage of the royalties paid to us by such sublicensee, subject to specified restrictions. We are obligated to make any such royalty payments until the later of: (i) a specified anniversary of the first commercial sale of the applicable product or (ii) the expiration of the last valid patent claim licensed by RICC under the RICC License Agreement underlying such product. Upon the occurrence of specified events, the

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royalty owed to RICC will be decreased by a specified percentage. During the year ended December 31, 2018, we incurred $0.7 million in expense related to a milestone reached, which is included in research and development expense in the accompanying consolidated statements of operations and comprehensive loss.

The RICC License Agreement will terminate upon the latest of the expiration of all of RICC's royalty rights, the termination of the last Miragen target or the expiration of its right to obtain a product license for a new target under the RICC License Agreement. We may also terminate the RICC License Agreement for convenience upon a specified number of days' prior notice to RICC, subject to specified terms and conditions. Either party may terminate the RICC License Agreement upon an uncured material breach by the other party and RICC may terminate the RICC License Agreement upon the occurrence of other specified events immediately or after such event is not cured within a specified number of days, as applicable.

All charges incurred under the RICC License Agreement have been expensed to date due to the uncertainty as to future economic benefit from the acquired rights.

During the years ended December 31, 2018 and 2017, we paid $0.3 million and $0.6 million, respectively, to RICC for raw materials to be used in our drug manufacturing process.

Subcontract Agreement with Yale University

In October 2014, together with Yale University, or Yale, we entered into a subcontract agreement and into a subaward agreement in March 2015, or the Yale Agreements, which was subsequently amended. Under the Yale Agreements, we are providing specified services regarding the development of a proprietary compound that targets miR-29 in the indication of IPF. Yale entered into the Yale Agreements in connection with a grant that Yale received from the NIH for the development of a miR-29 mimic as a potential therapy for pulmonary fibrosis.

In consideration of our services under the Yale Agreements, Yale has agreed to reimburse us a specified amount over five years, subject to the availably of funds under the grant and continued eligibility. Under the terms of the Yale Agreements, we retain all rights to any and all intellectual property developed solely by us in connection with the Yale Agreements. Yale has also agreed to provide us with an exclusive option to negotiate in good faith for an exclusive, royalty-bearing license from Yale for any intellectual property developed by Yale or jointly by the parties under the Yale Agreements. Yale is responsible for filing, prosecuting, and maintaining foreign and domestic patent applications and patents on all inventions jointly developed by the parties under the Yale Agreements. Through December 31, 2018, we received $0.9 million under the Yale Agreements.

The Yale Agreements terminate automatically on the date that Yale delivers its final research report to the NIH under the terms of the grant underlying the Yale Agreements. Each party may also terminate the Yale Agreements upon a specified number of days' notice if the NIH's grant funding is reduced or terminated or upon material breach by the other party.

License Agreements with the t2cure GmbH

In October 2010, we entered into a license and collaboration agreement, or the t2cure Agreement, with t2cure GmbH, or t2cure, which was subsequently amended. Under the t2cure Agreement, we received a worldwide, royalty bearing, and exclusive license to specified patent and technology rights relating to miR-92.

In consideration of rights granted by t2cure, we paid an upfront fee of $46 thousand and agreed to: (i) pay an annual license maintenance fee in the amount of €3 thousand ($3 thousand as of December 31, 2018) and (ii) reimburse t2cure for costs incurred in conjunction with the filing, prosecution, enforcement, and maintenance of patent rights.

Under the terms of the t2cure Agreement, we are obligated to make the following future milestone payments for each licensed product, as defined in the t2cure Agreement: (i) up to approximately $0.7 million upon the initiation of certain defined clinical trials; (ii) $2.5 million upon regulatory approval in the United States; and (iii) up to $1.5 million per region upon regulatory approval in the EU or Japan. Additionally, if we or any of our sublicensees successfully commercializes any product candidate subject to the t2cure Agreement, we are responsible for royalty payments equal to percentages in the low-single digits upon net sales of licensed products, and under specified circumstances, sublicense fees equal to a percentage in the low twenties of sublicense income received by us. We are obligated to make any such royalty payment until the later of: (i) the tenth anniversary of the first commercial sale of the applicable product or (ii) the expiration of the last valid claim to a patent licensed by t2cure under the t2cure Agreement covering such product. If such patent claims expire prior to the end of the ten-year term, then the royalty owed to t2cure will be decreased by a specified percentage. We also have the right to decrease our royalty payments by a specified percentage for royalties paid to third parties for licenses to certain third-party intellectual property.

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The license term extends on a country-by-country basis until the later of: (i) the tenth anniversary of the first commercial sale of a licensed product in a country, and (ii) the expiration of the last to expire valid claim that claims such licensed product in such country. Upon expiration of the royalty payment obligation, we will have a fully paid license in such country. We have the right to terminate the t2cure Agreement at will, on a country-by-country basis, after 60 days' written notice. The t2cure Agreement will also automatically terminate upon our bankruptcy or insolvency or upon notice of an uncured material breach.

All charges incurred under the t2cure Agreement have been expensed to date, due to the uncertainty as to future economic benefit from the acquired rights.

License Agreement with The Brigham and Women's Hospital

In May 2016, we entered into an exclusive patent license agreement, or the BWH License Agreement, with The Brigham and Women's Hospital, or BWH. Under the BWH License Agreement, we have an exclusive, worldwide license, including a right to sublicense, to specified patent rights and a nonexclusive, worldwide license, including a right to sublicense, to specified technology rights of BWH, each related to certain microRNAs believed to be involved in various neurodegenerative disorders. As consideration for these rights, we are obligated to pay a specified annual license fee. BWH is also entitled to milestone payments of up to approximately $2.6 million for each of our product candidates developed based on the patent rights subject to the BWH License Agreement plus a one-time sales milestone payment of $0.3 million for all product candidates developed based on the patent rights subject to the BWH License Agreement. If we successfully commercialize any product candidate subject to the BWH License Agreement, then BWH is entitled to royalty payments in the low-single digits on the net sales of such product. BWH's right to these royalty payments will expire on a product-by-product and country-by-country basis upon the expiration of the last patent claim in such country that is subject to the BWH License Agreement and covers the product, and our license to such product in such country will become fully paid at such time. BWH is also entitled to a percentage in the low-double digits of any sublicense income from such product, subject to specified exceptions. We are also responsible for all costs associated with the preparation, filing, prosecution, and maintenance of the patent rights subject to the BWH License Agreement. Additionally, we are obligated to use commercially-reasonable efforts to develop a product under the BWH License Agreement and to meet specified diligence milestones thereunder.

The BWH License Agreement will terminate upon the expiration of all issued patents and patent applications subject to the patent rights under the agreement. We may also terminate the BWH License Agreement for convenience upon a specified number of days' prior notice to BWH. BWH may terminate the BWH License Agreement upon a breach by us of our payment obligations and upon the occurrence of other specified events that are not cured within a specified number of days, provided that such termination is automatic upon our bankruptcy or insolvency.

Manufacturing

We do not own or operate manufacturing facilities for the production of cobomarsen, remlarsen, MRG-110, or other product candidates that we develop, nor do we have plans to develop our own manufacturing operations in the foreseeable future. We currently depend on third-party contract manufacturers for all of our required raw materials, active pharmaceutical ingredients, and finished product candidates for our clinical trials. We do not have any current contractual arrangements for the manufacture of commercial supplies of cobomarsen, remlarsen, MRG-110, or any other product candidates that we develop. We currently employ internal resources and third-party consultants to manage our manufacturing contractors.

Sales and Marketing

We have not yet defined our sales, marketing, or product distribution strategy for cobomarsen, remlarsen, MRG-110, or any of our other product candidates because our product candidates are still in preclinical or early-stage clinical development. Our commercial strategy may include the use of strategic partners, distributors, a contract sale force, or the establishment of our own commercial and specialty sales force. We plan to further evaluate these alternatives as we approach approval for one of our product candidates.

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Intellectual Property

We are actively building an intellectual property portfolio around our clinical-stage product candidates and discovery programs. A key component of this portfolio strategy is to seek patent protection in the United States and in major market countries that we consider important to the development of our business worldwide. As of December 31, 2018, we have a portfolio of 291 patents and applications of which 179 are issued or allowed and 112 are pending applications. This portfolio includes methods of use and composition patents, and patent applications on our three lead product candidates, cobomarsen, remlarsen, and MRG-110. Our success depends in part on our ability to obtain and maintain proprietary protection for our product candidates and other discoveries, inventions, trade secrets and know-how that are critical to our business operations. Our success also depends in part on our ability to operate without infringing the proprietary rights of others, and in part, on our ability to prevent others from infringing our proprietary rights. A comprehensive discussion on risks relating to intellectual property is provided under “Risk Factors” under the subsection “Risks Related to our Intellectual Property”.

We have filed patent applications directed to compositions of matter and methods of use covering cobomarsen in the United States and under the Patent Cooperation Treaty, or PCT, to access foreign countries. A U.S. patent application issued as U.S. 9,771,585 on September 26, 2017, which will expire in June of 2036 if we continue to pay the maintenance fees and annuities when due, with the possibility of Patent Term Extension that may be granted by the USPTO due to administrative delays in the FDA. Prior to the issue of this application, we filed a continuation application in August 2017 directed to methods of treatment, as U.S. 15/677,818, and this application is currently pending. We also filed an U.S. application directed to compositions of matter through the PCT, as U.S. 15/714,671, and this application issued as U.S. 9,994,852 on June 12, 2018, which will expire in June of 2036 if we continue to pay the maintenance fees and annuities when due. Prior to the issue of this application, we filed a continuation application in May 2018 as U.S. 15/976,333, and this application is currently pending.

We expect these pending applications will issue as U.S. patents in the next one to three years, with a projected expiration year of 2036 if we continue to pay the maintenance fees and annuities when due, with the possibility of additional terms from the USPTO prosecution delays and from patent term extensions that may be granted due to administrative delays in the FDA. We also have pending applications that cover methods of use of cobomarsen and related compositions. Collectively, these applications, if they issue, would have patent expirations from 2036 if we continue to pay the maintenance fees and annuities when due, not including any possible additional terms for patent term adjustments or patent term extensions. We do not know if any patent will issue from any of these applications and, if any issue, we do not know whether the issued patents will provide significant proprietary protection or commercial advantage against our competitors or generics. Even if they are issued, our patents may be circumvented, challenged, opposed, and found to be invalid or unenforceable.

We have filed patent applications directed to compositions of matter and methods of use covering MRG-110 in the U.S. and under the PCT, to access foreign countries. A patent directed to compositions of matter and methods of use of MRG-110 issued as U.S. 9,803,202, on October 31, 2017, and will expire in June 2033 if we continue to pay the maintenance fees and annuities when due, with the possibility of Patent Term Extension that may be granted by the USPTO due to administrative delays in the FDA. We also have issued patents and pending applications that cover various therapeutic uses and generic compositions of matter comprising MRG-110. Collectively, these patents and patent applications, if they issue, would have patent expirations ranging from 2028 to 2036 if we continue to pay the maintenance fees and annuities when due, not including any possible additional terms for patent term adjustments or patent term extensions. We do not know if any patent will issue from any of the pending applications and, if any issue, we do not know whether the issued patents will provide significant proprietary protection or commercial advantage against our competitors or generics. Even if they are issued, our patents may be circumvented, challenged, opposed, and found to be invalid or unenforceable.

We have filed patent applications directed to compositions of matter and methods of use covering remlarsen in the United States and under the PCT to access foreign countries. A U.S. patent application issued as U.S. 9,376,681 on June 28, 2016, which will expire in September of 2035 if we continue to pay the maintenance fees and annuities when due, with the possibility of Patent Term Extension that may be granted by the USPTO due to administrative delays in the FDA. Prior to the issue of this application, we filed a continuation application in June 2016 also directed to compositions of matter in the United States, as U.S. 15/175,636, and this application issued as U.S. 9,994,847 on June 12, 2018, which will expire in September of 2035, if we continue to pay the maintenance fees and annuities when due. Prior to the issue of this application, we filed a continuation application in June 2018, as U.S. 16/002,845, and this application is currently pending. We also have issued patents and pending applications that cover various therapeutic uses and generic compositions comprising remlarsen. Collectively, these patents and patent applications, if they issue, would have patent expirations ranging from 2028 to 2035 if we continue to pay the maintenance fees and annuities when due, not including any possible additional terms for patent term adjustments or patent term extensions. We do not know if any patent will issue from any of the pending applications and, if any issue, we do not know whether the issued patents will provide significant proprietary protection or commercial advantage against our

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competitors or generics. Even if they are issued, our patents may be circumvented, challenged, opposed, and found to be invalid or unenforceable.

For our earlier stage product candidates, we have filed compositions of matter and methods of use patent applications in the United States, and under the PCT to access foreign countries.

In addition to patent protection, we seek to rely on trade secret protection, trademark protection and know-how to expand our proprietary position around our chemistry, technology and other discoveries and inventions that we consider important to our business. We also seek to protect our intellectual property in part by entering into confidentiality agreements with our employees, consultants, scientific advisors, clinical investigators, and other contractors and also by requiring our employees, commercial contractors, and certain consultants and investigators, to enter into invention assignment agreements that grant us ownership of any discoveries or inventions made by them. Further, we seek trademark protection in the United States and internationally where available and when we deem appropriate. We have obtained registrations for the Miragen trademark, which we use in connection with our pharmaceutical research and development services as well as our clinical-stage product candidates. We currently have such registrations for Miragen in the United States, Canada, Japan, and the EU.

Competition

The biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprietary products. Our clinical and preclinical product candidates may address multiple markets. Ultimately, the diseases our product candidates target for which we may receive marketing authorization will determine our competition. We believe that for most or all of our product development programs, there will be one or more competing programs under development by other companies. Any products that we may commercialize will have to compete with existing therapies and new therapies that may become available in the future. We face potential competition from many different sources, including larger and better-funded biotechnology and pharmaceutical companies. In many cases, the companies with competing programs will have access to greater resources and expertise than we do and may be more advanced in those programs.

We believe that our current and future competition for resources and eventually for customers can be grouped into three broad categories:

- companies working to develop microRNA targeted products, including Regulus Therapeutics Inc. and InteRNA Technologies B.V.;
- companies working to develop other types of oligonucleotide therapeutic products, including Ionis Pharmaceuticals, Inc., Alnylam Pharmaceuticals, Inc., Arrowhead Pharmaceuticals, Inc., Dicerna Pharmaceuticals, Inc., STELLAS Life Sciences Group, Inc., Silence Therapeutics AG, and Translate Bio, Inc.; and
- companies with marketed products and development programs for therapeutics that treat the same diseases for which we may also be developing potential treatments.

The following companies have therapeutics marketed or in development for CTCL: Argenx, Bristol-Myers Squibb Company, Celgene Corporation, Helsinn Group, innate Pharma, Kyowa Hakko Kirin, Merck & Co., Inc., Mylan Pharmaceuticals Inc., Novartis International AG, Spectrum Pharmaceuticals, Inc., Seattle Genetics, Inc., Takeda Pharmaceutical Company Ltd, and Valeant Pharmaceuticals International, Inc.

The following companies have marketed therapeutics for pulmonary fibrosis: Boehringer Ingelheim GmbH, F. Hoffmann-La Roche Ltd.

We believe that the key competitive factors that will affect the success of any of our product candidates, if commercialized, are likely to be their efficacy, safety, convenience, price, and the availability of reimbursement from government and other third-party payors relative to such competing products. Our commercial opportunity could be reduced or eliminated if our competitors have products that are superior in one or more of these categories.

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Government Regulation

FDA Drug Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements at any time during the product development process may subject a company to a variety of administrative or judicial sanctions, such as imposition of clinical hold, FDA refusal to approve pending new drug applications, or NDAs, warning or untitled letters, withdrawal of approval, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

We cannot market a drug product candidate in the United States until the drug has received FDA approval. The steps required before a drug may be marketed in the United States generally include the following:

- completion of extensive preclinical laboratory tests, animal studies, and formulation studies in accordance with the FDA's good laboratory practices, or GLP, regulations;
- approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated at that site;
- submission to the FDA of an investigational new drug application, or IND, for human clinical testing, which must become effective before human clinical trials may begin;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice, or GCP, requirements to establish the safety and efficacy of the drug for each proposed indication;
- submission to the FDA of an NDA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA advisory committee review, if applicable
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the active pharmaceutical ingredient, or API, and finished drug product are produced and tested to assess compliance with current good manufacturing practices, or cGMPs; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Satisfaction of FDA pre-market approval requirements typically takes many years, and the actual time required may vary substantially based upon the type, complexity, and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation, and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including GLP. An IND sponsor must submit the results of preclinical testing to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin if all other requirements, including IRB review and approval, have been met. If the FDA raises concerns or questions about the conduct of the trial, such as whether human research subjects will be exposed to an unreasonable health risk, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical trials can proceed.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, including 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 the objectives of the trial, the

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parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. Each protocol and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an IRB for approval at each site at which the clinical trial will be conducted. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements or may impose other conditions. Information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on their www.clinicaltrials.gov website.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess pharmacological actions, side effects associated with increasing doses and, if possible, early evidence of effectiveness. Phase 2 usually involves trials in a limited patient population to study metabolism of the drug, pharmacokinetics, the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 clinical trials, also called pivotal trials, are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases, the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 clinical trial with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity, or prevention of a disease with a potentially serious outcome, and confirmation of the result in a second trial would be practically or ethically impossible.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the United States. The NDA must include the results of all preclinical, clinical, and other testing, and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting an NDA is substantial. The submission of most NDAs is additionally subject to a substantial application user fee, and the sponsor of an approved NDA is also subject to annual program fees. These fees are typically increased annually. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review typically takes twelve months from the date the NDA is submitted to FDA because the FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth 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. 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 may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an advisory committee, which is typically a panel that includes clinicians and other experts-for review, evaluation, and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving

an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with cGMPs is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. 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, require that post-approval studies, including Phase 4 clinical trials,

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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 and use restrictions or other risk management mechanisms under a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits of the drug outweigh the potential risks.

A REMS can include a medication guide, a communication plan for healthcare professionals and elements to assure safe use, such as special training and certification requirements for individuals who prescribe or dispense the drug, requirements that patients enroll in a registry, and other measures that the FDA deems necessary to assure the safe use of the drug. The requirement for a REMS can materially affect the potential market and profitability of the drug. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs. Such supplements are typically reviewed within 10 months of receipt by the FDA.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting an NDA or a biological license application, or BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full NDA or BLA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the application fee.

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

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for development and review of new drug products that meet certain criteria. Specifically, new drug products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug may request that the FDA

designate the drug as a Fast Track product at any time during the clinical development of the product. For a Fast Track-designated product, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted. If the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy

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exists or a significant improvement in the treatment, diagnosis, or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Drug 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, which means that they may be approved on the basis of adequate and well-controlled clinical trials establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or 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 that a sponsor of a drug product subject to accelerated approval perform adequate and well-controlled post-marketing clinical trials. In addition, the FDA currently requires, as a condition for accelerated approval, pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

In addition, the Breakthrough Therapy Designation is intended to expedite the development and review of products that treat serious or life-threatening diseases or conditions. 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, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the features of Fast Track designation, as well as more intensive FDA interaction and guidance. The Breakthrough Therapy Designation is distinct from both accelerated approval and priority review, but these can also be granted to the same product candidate if the relevant criteria are met. 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. Requests for breakthrough therapy designation will be reviewed within 60 days of receipt, and the FDA will either grant or deny the request.

Fast Track designation, priority review, accelerated approval, and breakthrough therapy designation do not change the standards for approval but may expedite the development or approval process by allowing for approval based on a surrogate endpoint likely to predict clinical benefit of the underlying drug, rather than through a direct measure of clinical benefit. Even if we receive one of these designations for our product candidates, the FDA may later decide that our product candidates no longer meet the conditions for qualification. In addition, these designations may not provide us with a material commercial advantage.

Post-Approval Requirements

Once an NDA is approved, a product may be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities, and promotional activities involving the internet and social media. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-approval testing, known as Phase 4 testing, REMS, surveillance to monitor the effects of an approved product, or restrictions on the distribution or use of the product. In addition, quality control, drug manufacture, packaging, and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money, and effort in the areas of production and quality control to

maintain compliance with cGMPs. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, 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 applications or supplements to approved applications or suspension or revocation of product approvals;

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- product seizure or detention, or refusal to permit the import or export of products, or injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. Drugs may be promoted 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.

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 and jurisdictions regarding quality, safety, and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales, and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable foreign regulatory authorities before we can commence clinical trials or marketing of the product in foreign countries and jurisdictions.

Some countries outside of the United States have a similar process that requires the submission of a clinical trial application, or CTA, much like the IND prior to the commencement of human clinical trials. In Europe, for example, a CTA must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country's requirements, a clinical trial may proceed in that country. To obtain regulatory approval to commercialize a new drug under EU regulatory systems, we must submit a marketing authorization application, or MAA. The MAA is similar to the NDA, with the exception of, among other things, country-specific document requirements.

In Canada, biopharmaceutical product candidates are regulated by the Food and Drugs Act and the rules and regulations promulgated thereunder, which are enforced by the Therapeutic Products Directorate of Health Canada, or TPD. Before commencing clinical trials in Canada, an applicant must complete preclinical studies and file a CTA with the TPD. After filing a CTA, the applicant must receive different clearance authorizations to proceed with Phase 1 clinical trials, which can then lead to Phase 2 and Phase 3 clinical trials. To obtain regulatory approval to commercialize a new drug in Canada, a new drug submission, or NDS, must be filed with the TPD. If the NDS demonstrates that the product was developed in accordance with the regulatory authorities' rules, regulations, and guidelines and demonstrates favorable safety and efficacy and receives a favorable risk/benefit analysis, the TPD issues a notice of compliance which allows the applicant to market the product.

Other Regulations

Although we currently do not have any products on the market, our current and future business operations may be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. Such laws or regulations include, without limitation, state, federal, and foreign anti-kickback, fraud and abuse, false claims, privacy and security, price reporting, and physician sunshine laws or regulations. Some of our pre-commercial activities are subject to some of these laws.

The federal Anti-Kickback Statute makes it illegal for any person or entity, including a prescription drug manufacturer or a party acting on its behalf, to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. 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, formulary managers, and beneficiaries on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions 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 exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. 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 Anti-Kickback Statute has been violated. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Violations of this law are punishable by up to five years in prison, and can also result in criminal fines, civil money penalties, and exclusion from participation in federal healthcare programs.

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Moreover, 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.

The federal civil False Claims Act prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent or not provided as claimed. Persons and entities can be held liable under these laws if they are deemed to "cause" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state, and third-party reimbursement for our products, and the sale and marketing of our products are subject to scrutiny under this law. Penalties for federal civil False Claims Act violations may include up to three times the actual damages sustained by the government, plus mandatory civil penalties for each separate false claim, the potential for exclusion from participation in federal healthcare programs, and, although the federal False Claims Act is a civil statute, False Claims Act violations may also implicate various federal criminal statutes.

The Health Insurance Portability and Accountability Act of 1996, or HIPAA, created additional federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, 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. Like the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

The civil monetary penalties statute imposes penalties against any person or entity that, among other things, 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.

Also, many states have similar fraud and abuse statutes or regulations that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs. Additionally, to the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, mandates, among other things, the adoption of uniform standards for the electronic exchange of information in common healthcare transactions, as well as standards relating to the privacy and security of individually identifiable health information, which require the adoption of administrative, physical, and technical safeguards to protect such 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, or obtain 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 and business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA, and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties.

The Physician Payments Sunshine Act imposes, among other things, annual reporting requirements for covered manufacturers for certain payments and other transfers of value provided to physicians and teaching hospitals, as well as certain ownership and investment interests held by physicians and their immediate family members. Failure to submit timely, accurately, and completely the required information for all payments and transfers of value and ownership or investment interests may result in civil monetary penalties. Certain states also mandate implementation of compliance programs, impose restrictions on drug manufacturer marketing practices, and/or require the tracking and reporting of gifts, compensation, and other remuneration to physicians.

Because we intend to commercialize products that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we intend to develop a comprehensive compliance program that establishes internal control to facilitate adherence to the rules and program requirements to which we will or may become subject. Although the development and implementation of compliance programs designed to establish internal control and facilitate compliance can mitigate the risk of investigation, prosecution, and penalties assessed for violations of these laws, the risks cannot be entirely eliminated.

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International data protection laws, including, without limitation, the EU's General Data Protection Regulation, or GDPR, and EU member state data protection legislation, also apply to health-related and other personal data that we process, including, without limitation, personal data relating to clinical trial participants in the EU. The GDPR imposes, significant obligations on controllers and processors of personal data, including, among other things, standards relating to the privacy and security of personal data, which require the adoption of administrative, physical, and technical safeguards to protect such information. These laws also include, without limitation, requirements for establishing an appropriate legal basis for processing personal data, transparency requirements related to communications with data subjects regarding the processing of their personal data, notification requirements to individuals about the processing of their personal data, an individual data rights regime, mandatory data breach notifications, limitations on the retention of personal data, increased requirements pertaining to health data, and strict rules and restrictions on the transfer of personal data outside of the EU, including to the United States. The GDPR also imposes obligations and required contractual provisions to be included in contracts between companies subject to the GDPR and their third-party processors that relate to the processing of personal data. The GDPR allows EU member states to make additional laws and regulations further limiting the processing of genetic, biometric or health data. Failure to comply with these laws, where applicable, can result in the imposition of significant regulatory fines and penalties.

If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, administrative, regulatory, civil and criminal penalties, damages, fines, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, individual imprisonment, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, the curtailment or restructuring of our operations, and exclusion from participation in federal and state healthcare programs, any of which could adversely affect our ability to operate our business and our financial results.

Health Reform

In the United States and foreign jurisdictions, there have been a number of legislative and regulatory changes to healthcare systems that could affect our future results of operations. There have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs.

In particular, the Affordable Care Act has had a significant impact on the healthcare industry. The Affordable Care Act was designed to expand coverage for the uninsured while at the same time containing overall healthcare costs. With regard to pharmaceutical products, among other things, the Affordable Care Act revised the definition of "average manufacturer price" for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices and imposed a significant annual fee on companies that manufacture or import certain branded prescription drug products.

Since its enactment, certain aspects of the Affordable Care Act have faced Congressional and judicial challenges. In January 2017, 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 the Affordable Care Act. 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, an Executive Order was signed, directing federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Congress also has considered subsequent legislation to repeal or replace elements of the Affordable Care Act. In the coming years, additional legislative and regulatory changes could be made to governmental health programs that could significantly impact pharmaceutical companies and the success of its product candidates.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. In August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. These included reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013, and, due to subsequent legislative amendments to the statute, will stay in effect through 2025 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Moreover, the Drug Supply Chain Security Act imposes obligations on manufacturers of pharmaceutical products, among others, related to product tracking and tracing. Among its requirements, manufacturers need to provide certain information

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regarding the drug product to individuals and entities to which product ownership is transferred, label drug product with a product identifier, and keep certain records regarding the drug product. The transfer of information to subsequent product owners by manufacturers will eventually be required to be done electronically. Manufacturers will also be required to verify that purchasers of the manufacturers' products are appropriately licensed. Further, manufacturers will have drug product investigation, quarantine, disposition, and notification responsibilities related to counterfeit, diverted, stolen, and intentionally adulterated products, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs.

Coverage and Reimbursement

Sales of our product candidates, once approved, will depend, in part, on the extent to which the costs of our products will be covered by third-party payors, such as government health programs, private health insurers and managed care organizations. Third-party payors generally decide which drugs they will cover and establish certain reimbursement levels for such drugs. 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. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of such products. Sales of our product candidates, and any future product candidates, will therefore depend substantially on the extent to which the costs of our product candidates, and any future product candidates, will be paid by third-party payors. Additionally, the market for our product candidates, and any future product candidates, will depend significantly on access to third-party payors' formularies without prior authorization, step therapy, or other limitations such as approved lists of treatments for which third-party payors provide coverage and reimbursement. Additionally, coverage and reimbursement for therapeutic products can differ significantly from payor to payor. One third-party payor's decision to cover a particular medical product or service does not ensure that other payors will also provide coverage for the medical product or service, or will provide coverage at an adequate reimbursement rate. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our products to each payor separately and will be a costly and time-consuming process.

Third-party payors are developing increasingly sophisticated methods of controlling healthcare costs and increasingly challenging the prices charged for medical products and services. Additionally, the containment of healthcare costs has become a priority of federal and state governments and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls and transparency requirements, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could limit our net revenue and results. If these third-party payors do not consider our products to be cost-effective compared to other therapies, they may not cover our products once approved as a benefit under their plans or, if they do, the level of reimbursement may not be sufficient to allow us to sell our products on a profitable basis. Decreases in third-party reimbursement for our products once approved or a decision by a third-party payor to not cover our products could reduce or eliminate utilization of our products and have an adverse effect on our sales, results of operations and financial condition. In

addition, state and federal healthcare reform measures have been and will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our products once approved or additional pricing pressures.

Employees

As of December 31, 2018, we employed 80 employees, of which 77 were full-time employees. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective bargaining arrangements. We consider our employee relations to be good.

Corporate Information

Our principal executive offices are located at 6200 Lookout Road, Boulder, CO 80301, and our telephone number is (720) 643-5200. Our corporate website address is www.miragen.com. Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and amendments to reports filed pursuant to Sections 13(a) and 15(d) of the Securities

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Exchange Act of 1934, as amended, or the Exchange Act, will be made available free of charge on our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the U.S. Securities and Exchange Commission, or the SEC. The contents of our website are not incorporated into this Annual Report and our reference to the URL for our website is intended to be an inactive textual reference only.

This Annual Report contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this Annual Report, including logos, artwork, and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other company.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our initial public offering in June 2014, (b) in which we have total annual gross revenue of at least $1.07 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeded $700.0 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period. We refer to the Jumpstart Our Business Startups Act of 2012 in this Annual Report as the "JOBS Act," and references to "emerging growth company" have the meaning associated with it in the JOBS Act.

ITEM 1A. RISK FACTORS

Our business, financial condition, and operating results may be affected by a number of factors, whether currently known or unknown, including but not limited to those described below. Any one or more of such factors could directly or indirectly cause our actual results of operations and financial condition to vary materially from past or anticipated future results of operations and financial condition. Any of these factors, in whole or in part, could materially and adversely affect our business, financial condition, results of operations, and stock price. The following information should be read in conjunction with Part II, Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the consolidated financial statements and related notes in Part II, Item 8, "Financial Statements and Supplementary Data" of this Annual Report.

Risks Related to Our Financial Condition and Capital Requirements

We have incurred losses since our inception, have a limited operating history on which to assess our business, and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a clinical-stage biopharmaceutical company with a limited operating history. We have incurred net losses in each year since Private Miragen's inception in 2006. During the years ended December 31, 2018 and 2017, net loss was $32.7 million and $26.5 million, respectively. As of December 31, 2018, we had an accumulated deficit of $126.3 million.

As of December 31, 2018, we had cash and cash equivalents of $32.6 million and short-term investments of $29.9 million. In February 2017, we received $40.7 million in gross proceeds through a common stock private placement. In March 2017, we entered into a Common Stock Sales Agreement, or the ATM Agreement, with Cowen and Company, LLC, or 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 $50.0 million through Cowen as our sales agent. Through March 14, 2019, we had sold, pursuant to the terms of the ATM Agreement, 1,260,975 shares of our common stock for aggregate net proceeds of approximately $10.3 million after deducting initial expenses for executing the "at the market offering"

and commissions to Cowen as sales agent. In February 2018, we entered into an underwriting agreement, or the Underwriting Agreement, with Jefferies LLC, Evercore Group L.L.C., and Deutsche Bank Securities Inc., as representatives of several underwriters, or the Underwriters, relating to our public offering. Pursuant to the Underwriting Agreement, in February 2018 we sold 7,414,996 shares of our common stock, which resulted in net proceeds of approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses payable by us. In August 2018, we entered into a Common Stock Purchase Agreement, or the LLS Stock Purchase Agreement, with LLS, for the sale of up to $5.0 million of shares of our common stock to LLS in a private placement, or the Offering. Under the terms of the LLS Stock Purchase Agreement, we expect to raise approximately $5.0 million in gross proceeds by selling shares of our common stock in five separate closings to LLS. The initial closing of the Offering was held on August 6, 2018. At the initial closing, we issued 150,987 shares of our common stock, which resulted in net proceeds of approximately $0.9 million after deducting expenses incurred in connection with the Offering. We believe that we have sufficient capital to fund our operations in the normal course of business and to meet our liquidity needs

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through the first quarter of 2020.

We will continue to require substantial additional capital to continue our clinical development and potential commercialization activities. Accordingly, we will need to raise substantial additional capital to continue to fund our operations. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our clinical development efforts. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates. Changing circumstances may cause us to consume capital significantly faster or slower than we currently anticipate. If we are unable to acquire additional capital or resources, we will be required to modify our operational plans to complete future milestones. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available financial resources sooner than we currently anticipate. We may be forced to reduce our operating expenses and raise additional funds to meet our working capital needs, principally through the additional sales of our securities or debt financings or entering into strategic collaborations.

We have devoted substantially all of our financial resources to identify, acquire, and develop our product candidates, including conducting clinical trials and providing general and administrative support for our operations. To date, we have financed our operations primarily through the sale of equity securities and convertible promissory notes. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We expect our losses to increase as our product candidates enter more advanced clinical trials. It may be several years, if ever, before we complete pivotal clinical trials or have a product candidate approved for commercialization. We expect to invest significant funds into the research and development of our current product candidates to determine the potential to advance these product candidates to regulatory approval.

If we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval, and our ability to achieve sufficient market acceptance, pricing, coverage and adequate reimbursement from third-party payors, and adequate market share for our product candidates in those markets. Even if we obtain adequate market share for our product candidates, because the potential markets in which our product candidates may ultimately receive regulatory approval could be very small, we may never become profitable despite obtaining such market share and acceptance of our products.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future and our expenses will increase substantially if and as we:

- continue the clinical development of our product candidates;
- continue efforts to discover and develop new product candidates;
- continue the manufacturing of our product candidates or increase volumes manufactured by third parties;
- advance our programs into larger, more expensive clinical trials;
- initiate additional preclinical studies or clinical trials for our product candidates;
- seek regulatory and marketing approvals and reimbursement for our product candidates;
- establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval and market for ourselves;

- seek to identify, assess, acquire, and/or develop other product candidates;
- make milestone, royalty, or other payments under third-party license agreements;
- seek to maintain, protect, and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel; and
- experience any delays or encounter issues with the development and potential for regulatory approval of our clinical and product candidates such as safety issues, manufacturing delays, clinical trial accrual delays, longer follow-up for

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planned studies or trials, additional major studies or trials, or supportive trials necessary to support marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

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

We have no products approved for commercialization and have never generated any revenue from product sales. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaborators, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize one or more of our product candidates. We do not anticipate generating revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

- completing research and development of our product candidates;
- obtaining regulatory and marketing approvals for our product candidates;
- manufacturing product candidates and establishing and maintaining supply and manufacturing relationships with third parties that are commercially feasible, meet regulatory requirements and our supply needs in sufficient quantities to meet market demand for our product candidates, if approved;
- marketing, launching, and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- gaining market acceptance of our product candidates as treatment options;
- addressing any competing products;
- protecting and enforcing our intellectual property rights, including patents, trade secrets, and know-how;
- negotiating favorable terms in any collaboration, licensing, or other arrangements into which we may enter;
- obtaining coverage and reimbursement or pricing for our product candidates that supports profitability; and
- attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Portions of our current pipeline of product candidates have been in-licensed from third parties, which make the commercial sale of such in-licensed products potentially subject to additional royalty and milestone payments to such third parties. We will also have to develop or acquire manufacturing capabilities or continue to contract with contract manufacturers in order to continue development and potential commercialization of our product candidates. For instance, if the costs of manufacturing our drug product are not commercially feasible and we will need to develop or procure our drug product in a commercially feasible manner in order to successfully commercialize any future approved product, if any. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

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

To the extent that we raise additional capital through the sale of equity, including pursuant to any sales under the ATM Agreement, the LLS Stock Purchase Agreement, convertible debt or other securities convertible into equity, the ownership interest of our stockholders will be diluted, and the terms of these new securities may include liquidation or other preferences that adversely affect rights of our stockholders. For instance, through March 14, 2019, we had sold, pursuant to the terms of the ATM Agreement, 1,260,975 shares of our common stock for aggregate net proceeds of approximately $10.3 million, and, in August 2018, we sold 150,987 shares of common stock to LLS under the LLS Stock Purchase Agreement for net proceeds of $0.9 million. We anticipate that we will continue to make sales of our common stock under the ATM Agreement and the LLS Stock Purchase Agreement from time to time into the foreseeable future, and we may sell shares of our common stock of up to $50.0 million and $5.0 million in aggregate value under the ATM Agreement and the LLS Stock Purchase Agreement, respectively. Sales under the ATM Agreement or the LLS Stock Purchase Agreement dilute the ownership interest of our

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stockholders and may cause the price per share of our common stock to decrease. Debt financing, if available, would likely involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, making additional product acquisitions, or declaring dividends. For instance, our loan and security agreement with Silicon Valley Bank limits our ability to enter into an asset sale, enter into any change of control, incur additional indebtedness, pay any dividends, or enter into specified transactions with our affiliates. If we raise additional funds through strategic collaborations or licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates or future revenue streams or grant licenses on terms that are not favorable to us. We cannot be assured that we will be able to obtain additional funding if and when necessary to fund our entire portfolio of product candidates to meet our projected plans. If we are unable to obtain funding on a timely basis, we may be required to delay or discontinue one or more of our development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on potential business opportunities, which could materially harm our business, financial condition, and results of operations.

We have also historically received funds directly or indirectly from state and federal government grants for research and development. The grants have been, and any future government grants and contracts we may receive may be, subject to the risks and contingencies set forth below under the risk factor titled “Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take specified actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition, and results of operations.” Although we might apply for government contracts and grants in the future, we cannot be certain that we will be successful in obtaining additional grants for any product candidates or programs.

Risks Related to the Development of Our Product Candidates

Clinical trials are costly, time consuming, and inherently risky, and we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Clinical development is expensive, time consuming, and involves significant risk. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of development. Events that may prevent successful or timely completion of clinical development include but are not limited to:

- inability to generate satisfactory preclinical, toxicology, or other in vivo or in vitro data or diagnostics to support the initiation or continuation of clinical trials;
- delays in reaching agreement on acceptable terms with contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- delays in obtaining required approvals from institutional review boards or independent ethics committees at each clinical trial site;
- failure to permit the conduct of a clinical trial by regulatory authorities;
- delays in recruiting eligible patients and/or subjects in our clinical trials;
- failure by clinical sites or CROs or other third parties to adhere to clinical trial requirements;

- failure by our clinical sites, CROs or other third parties to perform in accordance with the good clinical practices requirements of the FDA or applicable foreign regulatory guidelines;

- patients and/or subjects dropping out of our clinical trials;

- adverse events or tolerability or animal toxicology issues significant enough for the FDA or other regulatory agencies to put any or all clinical trials on hold;

- occurrence of adverse events associated with our product candidates;

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- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;

- the cost of clinical trials of our product candidates, including manufacturing costs;

- negative or inconclusive results from our clinical trials, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon development programs in other ongoing or planned indications for a product candidate; and

- delays in reaching agreement on acceptable terms with third-party manufacturers and the time to manufacture sufficient quantities of our product candidates acceptable for use in clinical trials.

Any inability to successfully complete clinical development and obtain regulatory approval for our product candidates could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional non-clinical studies and the results obtained from studying such new formulation may not be consistent with previous results obtained. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow competitors to develop and bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

The approach we are taking to discover and develop novel therapeutics that target microRNAs is unproven and may never lead to marketable products.

The scientific discoveries that form the basis for our efforts to discover and develop our product candidates are relatively recent. To date, neither we nor any other company has received regulatory approval to market therapeutics utilizing microRNA-targeted molecules. The scientific evidence to support the feasibility of developing drugs based on these discoveries is both preliminary and limited. Successful development of microRNA-targeted therapeutic products by us will require solving a number of issues, including providing suitable methods of stabilizing the therapeutic product and delivering it into target cells in the human body. In addition, any product candidates that we develop may not demonstrate in patients the chemical and pharmacological properties ascribed to them in laboratory and preclinical trials, and they may interact with human biological systems in unforeseen, ineffective, or even harmful ways. For instance, our clinical and preclinical data to date has not been fully validated and we have no way of knowing if, after validation, our clinical trial data will be complete and consistent. If we do not successfully develop and commercialize product candidates based upon this technological approach, we may not become profitable and the value of our capital stock may decline.

Further, our focus on microRNA technology for developing product candidates as opposed to multiple, more proven technologies for drug development, increases the risk associated with our business. If we are not successful in developing an approved product using microRNA technology, we may not be able to identify and successfully implement an alternative product development strategy. In addition, work by other companies pursuing similar technologies may encounter setbacks and difficulties that regulators and investors may attribute to our product candidates, whether appropriately or not.

Our microRNA-targeted therapeutic product candidates are based on a relatively novel technology, which makes it unusually difficult to predict the time and cost of development and the time and cost, or likelihood, of subsequently obtaining regulatory approval. To date, no microRNA-targeted therapeutics have been approved for marketing in the United States.

We have concentrated our research and development efforts to date on a limited number of product candidates based on our microRNA-targeted therapeutic platform and identifying our initial targeted disease indications. Our future

success depends on our successful development of viable product candidates. Only three of our product candidates, cobomarsen, remlarsen, and MRG-110, are in clinical development, and the remainder of our product candidates are in preclinical development. There can be no assurance that we will not experience problems or delays in developing our product candidates and that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved.

Additionally, the FDA, the European Medicines Agency, and other regulatory authorities, have relatively limited experience with microRNA-targeted therapeutics. No regulatory authority has granted approval to anyone, including us, to market or commercialize microRNA-targeted therapeutics, which may increase the complexity, uncertainty, and length of the regulatory review and approval process for our product candidates. If our product candidates fail to prove to be safe and effective, and commercially viable, our product candidate pipeline would have little, if any, value, which would have a material adverse effect on our business, financial condition, or results of operations.

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The clinical trial, product approval, and manufacturing requirements of the FDA, the European Medicines Agency, and other regulatory authorities, and the criteria these regulators use to evaluate the safety and efficacy of a product candidate, vary substantially according to the type, complexity, novelty, and intended use of the product candidate. The regulatory review and approval process for novel product candidates such as microRNA-targeted therapeutics can be more expensive and take longer than for other, better known or more extensively studied product candidates. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the EU or from other countries or regions of the world, or how long it will take to commercialize our product candidates, even if approved for marketing. Approvals by one regulatory agency may not be indicative of the likelihood of approval by other regulatory bodies. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations, and prospects may be harmed.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial viability of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or terminate clinical trials. They additionally may result in a delay of regulatory approval by the FDA or comparable foreign authorities, or, even in the instance that an affected product candidate is approved, may result in a restrictive drug label.

Our cobomarsen, remlarsen, and MRG-110 product candidates have been studied in only a limited number of patients with a confirmed diagnosis or healthy volunteers. The most common adverse events of any grade were injection site reactions, including pain, itchiness, redness, and swelling when compounds were delivered intradermally or subcutaneously. We may experience a higher rate or severity of adverse events and comparable or higher rates of discontinuation of trial participants in our future clinical trials. There is no guarantee that additional or more severe side effects will not be identified during ongoing or future clinical trials of our product candidates for current and other indications. Undesirable side effects and negative results for other indications may negatively impact the development and potential for approval of our product candidates for their proposed indications.

Additionally, even if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may withdraw approvals of such products;
- regulatory authorities may require additional warnings on the drug label;
- we may be required to create a REMS, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of a product candidate, even if approved, and could significantly harm our business, results of operations, and prospects.

Our product development program may not uncover all possible adverse events that patients who take our product candidates may experience. The number of subjects exposed to our product candidates and the average exposure time in the clinical development program may be inadequate to detect rare adverse events that may only be detected once the product is administered to more patients and for greater periods of time.

Clinical trials by their nature utilize a sample of the potential patient population. However, with a limited number of subjects and limited duration of exposure, we cannot be fully assured that rare and severe side effects of our product candidates will be uncovered. Such rare and severe side effects may only be uncovered with a significantly larger number of patients exposed to the drug. If such safety problems occur or are identified after our product candidates reach the market, the FDA may require that we amend the labeling of the product or recall the product or may even withdraw approval for the product.

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Our microRNA-targeted therapeutic approach is novel. Negative public opinion and increased regulatory scrutiny of microRNA or other nucleic acid-based therapies may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

MicroRNA therapy remains a novel technology, with no microRNA-targeted therapeutic product approved to date in the United States. Public perception may be influenced by claims that microRNA therapy is unsafe, and microRNA therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of the diseases targeted by our product candidates, prescribing therapies that involve the use of our product candidates in lieu of, or in addition to, existing therapies with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion regarding microRNA or other nucleic acid-based therapeutics could have an adverse effect on our business, financial condition, or results of operations and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. SAEs in microRNA clinical trials for our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product candidates. For instance, in June 2016, the FDA placed a regulatory hold on the clinical trial of a microRNA- or nucleic acid-focused biopharmaceutical company with a microRNA-targeted product candidate for the treatment of hepatitis C virus due to SAEs in that trial. This company also voluntarily halted a Phase 1 clinical trial in patients with kidney disease due to unexpected toxicity issues in July 2018. Another microRNA-focused biopharmaceutical company also voluntarily halted an ongoing Phase 1 clinical trial for a microRNA-targeted therapy for multiple cancers in September 2016 due to multiple immune-related severe adverse events. We cannot predict what effect, if any, these clinical holds will have on the government and public perception of our product candidates.

We are heavily dependent on the success of our product candidates, which are in the early stages of clinical development. Some of our product candidates have produced results only in non-clinical settings, or for other indications than those for which we contemplate conducting development and seeking FDA approval, and we cannot give any assurance that we will generate data for any of our product candidates sufficiently supportive to receive regulatory approval in our planned indications, which will be required before they can be commercialized.

We have invested substantially all of our effort and financial resources to identify, acquire, and develop our portfolio of product candidates. Our future success is dependent on our ability to successfully further develop, obtain regulatory approval for, and commercialize one or more product candidates. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a product candidate.

We currently have three product candidates in clinical trials. Of these product candidates, cobomarsen has been predominantly administered in patients with the mycosis fungoides form of CTCL, or MF. This is only one of the multiple indications for which we plan to develop this product candidate. Additionally, our clinical and preclinical data to date is not validated, and we have no way of knowing if after validation our clinical trial data will be complete and consistent. There can be no assurance that the data that we develop for our product candidates in our planned indications will be sufficiently supportive to obtain regulatory approval.

Based on discussions with the FDA, we believe the results from the SOLAR clinical trial could potentially allow us to apply for accelerated approval in the United States. We cannot guarantee that the outcome of this Phase 2 clinical trial will be sufficient to support, or if the FDA will grant us, accelerated approval of cobomarsen. If our data is not supportive of accelerated approval of cobomarsen, we cannot predict when, if ever, we will be able to seek approval of cobomarsen.

In addition, none of our other product candidates have advanced into a pivotal clinical trial for our proposed indications, and it may be years before any such clinical trial is initiated and completed, if at all. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

Product development involves a lengthy and expensive process with an uncertain outcome, and results of earlier preclinical studies and clinical trials may not be predictive of future clinical trial results.

Clinical testing is expensive and generally takes many years to complete, and the outcome is inherently uncertain. Failure can

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occur at any time during the clinical trial process. Additionally, microRNAs are a new class of drug target and as such may have some potentially unknown risks from both an efficacy and safety perspective. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of larger, later-stage controlled clinical trials. Product candidates that have shown promising results in early-stage clinical trials may still suffer significant setbacks in subsequent clinical trials. Our clinical trials to date have been conducted on a small number of patients or healthy volunteers in limited numbers of clinical sites for a limited number of indications. We will have to conduct larger, well-controlled trials in our proposed indications to verify the results obtained to date and to support any regulatory submissions for further clinical development. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles despite promising results in earlier, smaller clinical trials. For instance, in June 2016, the FDA placed a regulatory hold on the clinical trial of a microRNA-focused biopharmaceutical company with a microRNA product candidate for the treatment of hepatitis C virus due to SAEs in that trial. This company also voluntarily halted a Phase 1 clinical trial in patients with kidney disease due to unexpected toxicity issues in July 2018. Another microRNA-focused biopharmaceutical company also voluntarily halted an ongoing Phase 1 clinical trial for a microRNA therapy for multiple cancers in September 2016 due to multiple immune-related severe adverse events. Moreover, clinical data are often susceptible to varying interpretations and analyses. We do not know whether any Phase 2, Phase 3, or other clinical trials we are conducting or may conduct will demonstrate consistent or adequate efficacy and safety with respect to the proposed indication for use sufficient to receive regulatory approval or market our drug candidates.

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and human resources, we may forego or delay pursuit of opportunities with some programs or product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or more profitable market opportunities. Our spending on current and future research and development programs and future product candidates for specific indications may not yield any commercially viable products. We may also enter into additional strategic collaboration agreements to develop and commercialize some of our programs and potential product candidates in indications with potentially large commercial markets. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaborations, licensing, or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

We may find it difficult to enroll patients in our clinical trials given the limited number of patients who have the diseases for which our product candidates are being studied. Difficulty in enrolling patients could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is essential to our success. The timing of our clinical trials depends in part on the rate at which we can recruit patients to participate in clinical trials of our product candidates, and we may experience delays in our clinical trials if we encounter difficulties in enrollment.

The eligibility criteria of our planned clinical trials may further limit the available eligible trial participants as we expect to require that patients have specific characteristics that we can measure or meet the criteria to assure their conditions are appropriate for inclusion in our clinical trials. For instance, we plan to enroll approximately 65 patients

per treatment group in our SOLAR trial of cobomarsen in patients with MF, but have not yet dosed our first patient. The estimated prevalence of MF is 16,000 to 20,000 cases in the United States and only a subset of this group will satisfy the enrollment criteria for our SOLAR trial. As a result, we cannot guarantee that we will be able to enroll a sufficient number of patients to complete the SOLAR trial in a timely manner. We may not be able to identify, recruit, and enroll a sufficient number of patients to complete our clinical trials in a timely manner because of the perceived risks and benefits of the product candidate under study, the availability and efficacy of competing therapies and clinical trials, and the willingness of physicians to participate in our planned clinical trials. If patients are unwilling to participate in our clinical trials for any reason, the timeline for conducting trials and obtaining regulatory approval of our product candidates may be delayed.

If we experience delays in the completion of, or termination of, any clinical trials of our product candidates, the commercial prospects of our product candidates could be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical trials would likely increase our overall costs, impair product candidate development, and jeopardize our ability to obtain regulatory approval relative to our current plans. Any of these occurrences may harm our business, financial condition, and prospects significantly.

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We may face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our approved products, if any, or product candidates harm patients, or is perceived to harm patients even when such harm is unrelated to our approved products, if any, or product candidates, our regulatory approvals, if any, could be revoked or otherwise negatively impacted, and we could be subject to costly and damaging product liability claims. If we are unable to obtain adequate insurance or are required to pay for liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage, a material liability claim could adversely affect our financial condition.

The use or misuse of our product candidates in clinical trials and the sale of any products for which we may obtain marketing approval exposes us to the risk of potential product liability claims. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. Some of our microRNA-targeted therapeutic candidates have shown adverse events in clinical trials, including injection site reactions and pain at the injection site, erythema, nausea, diarrhea, decreased white blood cell and platelet counts, neutropenia, elevated aspartate aminotransferase, alanine aminotransferase, uric acid, and creatine kinase levels, prolonged partial thromboplastin time, blurred vision, itchiness, fatigue, headache, and microscopic hematuria, among others. In almost all cases, these events were mild to moderate and self-limited. There is a risk that our future product candidates may induce similar or more severe adverse events. Patients with the diseases targeted by our product candidates may already be in severe and advanced stages of disease and have both known and unknown significant preexisting and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may or may not be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact, or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which an adverse event is unrelated to our product candidates, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may delay our regulatory approval process or impact and limit the type of regulatory approvals our product candidates receive or maintain.

As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition, or results of operations.

Although we have product liability insurance, which covers our clinical trials in the United States, for up to $5.0 million per occurrence, up to an aggregate limit of $5.0 million, our insurance may be insufficient to reimburse us for any expenses or losses we may suffer. We will also likely be required to increase our product liability insurance coverage for the advanced clinical trials that we plan to initiate. If we obtain marketing approval for any of our product candidates, we will need to expand our insurance coverage to include the sale of commercial products. There is no way to know if we will be able to continue to obtain product liability coverage and obtain expanded coverage, if we require it, in sufficient amounts to protect us against losses due to liability, on acceptable terms, or at all. We may not have sufficient resources to pay for any liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage. Where we have provided indemnities in favor of third parties under our agreements with them, there is also a risk that these third parties could incur liability and bring a claim under such indemnities. An individual may bring a product liability claim against us alleging that one of our product candidates causes, or is claimed to have caused, an injury or is found to be unsuitable for consumer use. Any such product liability claims may include allegations of defects in manufacturing, defects in design, failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. Any product liability claim brought against us, with or without merit, could result in:

- withdrawal of clinical trial volunteers, investigators, patients or trial sites, or limitations on approved indications;

- the inability to commercialize, or if commercialized, decreased demand for, our product candidates;
- if commercialized, product recalls, labeling, marketing or promotional restrictions, or the need for product modification;
- initiation of investigations by regulators;
- loss of revenues;
- substantial costs of litigation, including monetary awards to patients or other claimants;
- liabilities that substantially exceed our product liability insurance, which we would then be required to pay ourselves;

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- an increase in our product liability insurance rates or the inability to maintain insurance coverage in the future on acceptable terms, if at all;
- the diversion of management's attention from our business; and
- damage to our reputation and the reputation of our products and our technology.

Product liability claims may subject us to the foregoing and other risks, which could have a material adverse effect on our business, financial condition, or results of operations.

We may not be able to develop or identify a technology that can effectively deliver our product candidates to the intended diseased cells or tissues, and any failure in such delivery technology could adversely affect and delay the development of any or all of our other product candidates.

In connection with our clinical trials of cobomarsen, remlarsen, and MRG-110, we have used various routes of administration, including intravenous, intralesional, subcutaneous, and intradermal injections. While we have observed in our clinical trials that some or all of these routes of administration may be effective in delivering adequate levels of our product candidates to produce a therapeutic response, we cannot guarantee that this will be the case in any current or future clinical trials of our product candidates. If we fail to develop effective routes of delivery to the target diseased cells or tissues, such failure could adversely affect and delay the development of our product candidates.

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

A potential breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek a breakthrough therapy designation from the FDA for some of our product candidates. A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and for which preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For drugs or biological products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA could also be eligible for priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one of our product candidates is designated as a breakthrough therapy, the FDA may later decide that the product candidate no longer meets the conditions for designation and the designation may be rescinded.

We may seek Fast Track designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process.

If a product candidate is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA Fast Track designation. If we seek Fast Track designation for a product candidate, we may not receive it from the FDA. However, even if we receive Fast Track designation, Fast Track designation does not ensure that we will receive marketing approval in any particular timeframe or at all. We may not experience a faster development or regulatory review or approval process with Fast Track designation compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA's priority review procedures.

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We may attempt to obtain accelerated approval of our product candidates. If we are unable to obtain accelerated approval, we may be required to conduct clinical trials beyond those that we contemplate, or the size and duration of our pivotal clinical trials could be greater than currently planned, which could increase the expense of obtaining, reduce the likelihood of obtaining, and/or delay the timing of obtaining necessary marketing approvals. Even if we receive accelerated approval from the FDA, the FDA may require that we conduct confirmatory trials to verify clinical benefit. If our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-approval requirements, the FDA may seek to withdraw accelerated approval.

We may seek accelerated approval for our product candidates, including cobomarsen. The FDA may grant accelerated approval to a product designed to treat a serious or life-threatening condition that provides meaningful therapeutic advantage over available therapies and demonstrates an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease. If granted, accelerated approval may be contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's predicted effect on irreversible morbidity or mortality or other clinical benefit. The FDA may require that any such confirmatory study be initiated or substantially underway prior to the submission of an application for accelerated approval. If such post-approval studies fail to confirm the drug's clinical benefits relative to its risks, the FDA may withdraw its approval of the drug. If we choose to pursue accelerated approval, there can be no assurance that the FDA will agree that our proposed primary endpoint is an appropriate surrogate endpoint. Similarly, there can be no assurance that after subsequent FDA feedback that we will continue to pursue accelerated approval or any other form of expedited development, review, or approval, even if we initially decide to do so. Furthermore, if we submit an application for accelerated approval, there can be no assurance that such application will be accepted or that approval will be granted on a timely basis, or at all. The FDA also could require us to conduct further studies or trials prior to considering our application or granting approval of any type. We might not be able to fulfill the FDA's requirements in a timely manner, which would cause delays, or approval might not be granted because our submission is deemed incomplete by the FDA. A failure to obtain accelerated approval or any other form of expedited development, review or approval for a product candidate would result in a longer time period to commercialize such product candidate, could increase the cost of development of such product candidate, and could harm our competitive position in the marketplace.

Even if we receive accelerated approval from the FDA, we will be subject to rigorous post-approval requirements, including submission to the FDA of all promotional materials prior to their dissemination. The FDA may require us to conduct a confirmatory study to verify the predicted clinical benefit. The FDA could withdraw accelerated approval for multiple reasons, including our failure to conduct any required post-approval study with due diligence, or the inability of such study to confirm the predicted clinical benefit.

A failure to obtain accelerated approval or any other form of expedited review or approval for a product candidate could result in a longer time period prior to commercializing such product candidate, increase the cost of development of such product candidate, and harm our competitive position in the marketplace.

Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory requirements.

If any of our product candidates are approved, we will be subject to ongoing regulatory requirements with respect to manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing clinical trials, and submission of safety, efficacy, and other post-approval information, including both federal and state requirements in the United States, and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to continuously comply with FDA and comparable foreign requirements, including ensuring that quality control and manufacturing procedures conform to current cGMPs, regulations and corresponding foreign regulatory manufacturing requirements. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMPs and adherence to commitments made in any new drug application or MAA.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the marketed product. We will be required to report adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation could result in delays in product development or commercialization, or increased costs to assure compliance. If our original marketing approval for a product candidate was granted accelerated approval by the FDA, we could be required to conduct a successful post-marketing clinical trial in order to confirm the

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clinical benefit of our products. An unsuccessful post-marketing clinical trial or failure to complete such a trial could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, the regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities; or
- require a product recall.

Any government investigation of alleged violations of law would be expected to require us to expend significant time and resources in response and could generate adverse publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to develop and commercialize our products, and the value of the company and our operating results would be adversely affected.

Moreover, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. 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 civil, criminal, and administrative penalties.

In addition, if we were able to obtain accelerated approval of any of our drug candidates, the FDA may require us to conduct a confirmatory study to verify the predicted clinical benefit. Other regulatory authorities outside of the United States, may have similar requirements. The results from the confirmatory study may not support the clinical benefit, which could result in the approval being withdrawn. While operating under accelerated approval, we will be subject to certain restrictions that we would not be subject to upon receiving regular approval.

Healthcare legislative reform measures may have a material adverse effect on our business, financial condition, or results of operations.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, or collectively the ACA, was passed, which was intended to substantially change the way healthcare is financed by both governmental and private insurers, and significantly impact the U.S. pharmaceutical industry. The ACA, among other things, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled,

infused, instilled, implanted, or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of specified branded prescription drugs, and promotes a new Medicare Part D coverage gap discount program.

Since the ACA's enactment, there have been, and continue to be, Congressional, executive branch, judicial, and regulatory challenges to the ACA, and both Congress and President Trump have delayed implementation or effectively repealed some of the ACA's requirements through legislation, Executive Orders, failures to fund, and other actions. Additionally, on December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Cuts and Jobs Act of 2017, or the Tax Act. While the U.S. Texas District Court Judge, as well as the Trump Administration and CMS, have stated that the ruling will have no immediate effect

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pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA and our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted, and we expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand or lower pricing for our product candidates or additional pricing pressures.

We may be subject, directly or indirectly, to foreign, federal, and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties, sanctions, or other liability.

Our operations may be subject to various foreign, federal, and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and Physician Payments Sunshine Act, the GDPR, and other regulations. These laws may impact, among other things, our relationships with healthcare professionals and our proposed sales, marketing, and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering, or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;

- federal civil and criminal false claims laws and civil monetary penalties law, including the federal False Claims Act, and can be enforced by individuals through civil whistleblower or qui tams actions, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;

- the Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit, among other things, executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their implementing regulations, which imposes specified obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information without the appropriate authorization, on entities subject to the law, such as certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, and their respective business associates, individuals, and entities that perform services for them that involve the creation, use, maintenance, or disclosure of individually identifiable health information;

- the federal Physician Payment Sunshine Act under the ACA which requires manufacturers of drugs, devices, biologics, and medical supplies, with certain exceptions, to report annually to the Centers for Medicare and Medicaid Services, or CMS, information related to payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers, as well as their immediate family members and applicable group purchasing organizations;

- the GDPR and other EU member state data protection legislation, which requires data controllers and processors, to adopt administrative, physical, and technical safeguards to protect personal data, including health-related data,

including mandatory contractual terms with third-party providers, requirements for establishing an appropriate legal basis for processing personal data, transparency requirements related to communications with data subjects regarding the processing their personal data, standards for obtaining consent from individuals to process their personal data, notification requirements to individuals about the processing of their personal data, an individual data rights regime, mandatory data breach notifications, limitations on the retention of personal data, increased requirements pertaining to health data, and strict rules and restrictions on the transfer of personal data outside of the EU, including to the United States; and

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws that may apply to items or services reimbursed by any third-party payor, including governmental and private payors, to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by

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the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; 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; state and local laws that require the registration of pharmaceutical sales representatives; and state laws governing the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent healthcare reform legislation has strengthened these laws. For example, the ACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute and criminal healthcare fraud statutes, such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate the law in order to have committed a violation. Moreover, the ACA 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 False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil, criminal, and administrative penalties, disgorgement, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, individual imprisonment, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, 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.

Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take specified actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition, and results of operations.

During the course of our development of our product candidates, we have been funded in part through federal and state grants, including but not limited to the funding we received from Yale pursuant to a subcontract agreement with Yale. In addition to the funding we have received to date, we have applied and intend to continue to apply for federal and state grants to receive additional funding in the future. Contracts and grants funded by the U.S. government, state governments and their related agencies include provisions that reflect the government’s substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

- require repayment of all or a portion of the grant proceeds, in specified cases with interest, in the event we violate specified covenants pertaining to various matters that include a failure to achieve;
- specify milestones or terms relating to use of grant proceeds, or to comply with specified laws;
- terminate agreements, in whole or in part, for any reason or no reason;
- reduce or modify the government’s obligations under such agreements without the consent of the other party;
- claim rights, including intellectual property rights, in products and data developed under such agreements;
- audit contract related costs and fees, including allocated indirect costs;

- suspend the contractor or grantee from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;

- impose U.S. manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;

- impose qualifications for the engagement of manufacturers, suppliers, and other contractors as well as other criteria for reimbursements;

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- suspend or debar the contractor or grantee from doing future business with the government;
- control and potentially prohibit the export of products;
- pursue criminal or civil remedies under the federal False Claims Act, False Statements Act, and similar remedy provisions specific to government agreements; and
- limit the government's financial liability to amounts appropriated by the U.S. Congress on a fiscal year basis, thereby leaving some uncertainty about the future availability of funding for a program even after we have been funded for an initial period.

In addition to those powers set forth above, the government funding we may receive could also impose requirements to make payments based upon sales of our products, if any, in the future.

We may not have the right to prohibit the U.S. government from using specified technologies developed by it, and we may not be able to prohibit third-party companies, including our competitors, from using those technologies in providing products and services to the U.S. government. The U.S. government generally takes the position that we have the right to royalty-free use of technologies that are developed under U.S. government contracts. These and other provisions of government grants may also apply to intellectual property we license now or in the future.

In addition, government contracts and grants normally contain additional requirements that may increase our costs of doing business, reduce our profits, and expose us to liability for failure to comply with these terms and conditions. These requirements include, for example:

- specialized accounting systems unique to government contracts and grants;
- mandatory financial audits and potential liability for price adjustments or recoupment of government funds after such funds have been spent;
- public disclosures of some contract and grant information, which may enable competitors to gain insights into our research program; and
- mandatory socioeconomic compliance requirements, including labor standards, non-discrimination and affirmative action programs, and environmental compliance requirements.

If we fail to maintain compliance with any such requirements that may apply to us now or in the future, we may be subject to potential liability and to termination of our contracts.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on our business, financial condition, or results of operations.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts, and business operations, and cause environmental

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

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Failure to comply with existing or future laws and regulations related to privacy or data security could lead to government enforcement actions (which could include civil or criminal fines or penalties), private litigation, other liabilities, and/or adverse publicity. Compliance or the failure to comply with such laws could increase the costs of our products and services, could limit their use or adoption, and could otherwise negatively affect our operating results and business.

Regulation of data processing is evolving, as federal, state, and foreign governments continue to adopt new, or modify existing, laws and regulations addressing data privacy and security, and the collection, processing, storage, transfer, and use of data. We and our partners may be subject to current, new, or modified federal, state, and foreign data protection laws and regulations (e.g., laws and regulations that address data privacy and data security, including, without limitation, health data). These new or proposed laws and regulations are subject to differing interpretations and may be inconsistent among jurisdictions, and guidance on implementation and compliance practices are often updated or otherwise revised, which adds to the complexity of processing personal data. These and other requirements could require us or our partners to incur additional costs to achieve compliance, limit our competitiveness, necessitate the acceptance of more onerous obligations in our contracts, restrict our ability to use, store, transfer, and process data, impact our or our partners' ability to process or use data in order to support the provision of our products or services, affect our or our partners' ability to offer our products and services in certain locations, or cause regulators to reject, limit, or disrupt our clinical trial activities.

In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure, and protection of health-related and other personal information could apply to our operations or the operations of our partners. In addition, we may obtain health information from third parties (including research institutions from which we may obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. Depending on the facts and circumstances, we could be subject to criminal penalties, including if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

International data protection laws, including, without limitation, the EU's GDPR that took effect in May 2018, and member state data protection legislation, may also apply to health-related and other personal information obtained outside of the United States. These laws impose strict obligations on the ability to process health-related and other personal information of data subjects in the EU, including in relation to use, collection, analysis, and transfer of such personal information. These laws include several requirements relating to obtaining the consent of the individuals to whom the personal data relates, limitations on data processing, establishing a legal basis for processing, notification of data processing obligations or security incidents to appropriate data protection authorities or data subjects, the security and confidentiality of the personal data and various rights that data subjects may exercise.

The GDPR prohibits the transfer, without an appropriate legal basis, of personal data to countries outside of the European Economic Area, or EEA, such as the United States, which are not considered by the European Commission to provide an adequate level of data protection. Switzerland has adopted similar restrictions. Although there are legal mechanisms to allow for the transfer of personal data from the EEA and Switzerland to the United States, uncertainty about compliance with EU data protection laws remains and such mechanisms may not be available or applicable with respect to the personal data processing activities necessary to research, develop, and market our products and services. For example, ongoing legal challenges in Europe to the mechanisms allowing companies to transfer personal data from the EEA to the United States could result in further limitations on the ability to transfer personal data across borders, particularly if governments are unable or unwilling to reach new or maintain existing agreements that support cross-border data transfers, such as the EU-U.S. and Swiss-U.S. Privacy Shield framework. Additionally, other countries have passed or are considering passing laws requiring local data residency and/or restricting the international transfer of data.

Under the GDPR, regulators may impose substantial fines and penalties for non-compliance. Companies that violate the GDPR can face fines of up to the greater of 20 million Euros or 4% of their worldwide annual turnover (revenue). The GDPR has increased our responsibility and liability in relation to personal data that we process, requiring us to put in place additional mechanisms to ensure compliance with the GDPR and other EU and international data protection rules.

Failure to comply with U.S. and international data protection laws and regulations could result in government enforcement actions (which could include civil or criminal penalties, fines or sanctions), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, patients about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations related to security or privacy, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. Compliance with data protection laws

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may be time-consuming, require additional resources and could result in increased expenses, reduce overall demand for our products and services and make it more difficult to meet expectations of or commitments to customers or partners.

Any of these matters could materially adversely affect our business, financial condition, or operational results.

Risks Related to Our Intellectual Property

We may not be successful in obtaining or maintaining necessary rights to microRNA targets, product compounds and processes for our development pipeline through acquisitions and in-licenses.

Presently, we have rights to the intellectual property, through licenses from third parties and under patents and patent applications that we own, to modulate only a subset of the known microRNA targets. Because our programs may involve a range of microRNA targets, including targets that require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license, or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities.

For example, we have previously collaborated and may continue to collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide an option to negotiate a license to any of the institution’s rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to it. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to third-party intellectual property rights, our business, financial condition, and prospects for growth could suffer.

We intend to rely on patent rights for our product candidates and any future product candidates. If we are unable to obtain or maintain exclusivity from the combination of these approaches, we may not be able to compete effectively in our markets.

We rely or will rely upon a combination of patents, trade secret protection, and confidentiality agreements to protect the intellectual property related to our technologies and product candidates. Our success depends in large part on our and our licensors’ ability to obtain regulatory exclusivity and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technologies and product candidates.

We have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our technologies and product candidates that are important to our business. This 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.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unsolved. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue, and even if such patents cover our product candidates, third parties may challenge their validity, enforceability, or scope, which may result in such patents being narrowed, found unenforceable, or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

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We, independently or together with our licensors, have filed several patent applications covering various aspects of our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent, or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If we cannot obtain and maintain effective protection of exclusivity from our regulatory efforts and intellectual property rights, including patent protection or data exclusivity, for our product candidates, we may not be able to compete effectively, and our business and results of operations would be harmed.

We may not have sufficient patent term protections for our product candidates to effectively protect our business.

Patents have a limited term. In the United States, the statutory expiration of a patent is generally 20 years after it is filed. Additional patent terms may be available through a patent term adjustment process, resulting from the United States Patent and Trademark Office, or USPTO, delays during prosecution. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from generic medications.

Patent term extensions under the Hatch-Waxman Act in the United States and under supplementary protection certificates in Europe may be available to extend the patent or data exclusivity terms of our product candidates. We will likely rely on patent term extensions, and we cannot provide any assurances that any such patent term extensions will be obtained and, if so, for how long. As a result, we may not be able to maintain exclusivity for our product candidates for an extended period after regulatory approval, if any, which would negatively impact our business, financial condition, results of operations, and prospects. If we do not have sufficient patent terms or regulatory exclusivity to protect our product candidates, our business and results of operations will be adversely affected.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products, and 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.

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming, and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in specified circumstances and weakened the rights of patent owners in specified situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

The USPTO has issued subject matter eligibility guidance to patent examiners instructing USPTO examiners on the ramifications of the Supreme Court rulings in Mayo Collaborative Services v. Prometheus Laboratories, Inc. and Association for Molecular Pathology v. Myriad Genetics, Inc., and applied the Myriad ruling to natural products and principles including all naturally occurring nucleic acids. In addition, the USPTO continues to provide updates to its guidance and this is a developing area. The USPTO guidance may make it impossible for us to pursue similar patent

claims in patent applications we may prosecute in the future.

Our patent portfolio contains claims of various types and scope, including chemically modified mimics, inhibitors, as well as methods of medical treatment. The presence of varying claims in our patent portfolio significantly reduces, but may not eliminate, our exposure to potential validity challenges.

For our U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law. 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 will be prosecuted and may also affect patent litigation. The USPTO has promulgated regulations and developed 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, did not come into effect until

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March 16, 2013. Accordingly, it is not yet 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, financial condition, or results of operations.

An important change introduced by the Leahy-Smith Act is that, as of March 16, 2013, the United States transitioned to a "first-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to either: (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our patents or patent applications.

Among some of the other changes introduced by the Leahy-Smith Act are changes that limit where a patentee may file a patent infringement suit and new procedures providing opportunities for third parties to challenge any issued patent in the USPTO. Included in these new procedures is a process known as Inter Partes Review, or IPR, which has been generally used by many third parties over the past four years to invalidate patents. The IPR process is not limited to patents filed after the Leahy-Smith Act was enacted and would therefore be available to a third party seeking to invalidate any of our U.S. patents, even those filed before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal court necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Additionally, the rights of review and appeal for IPR decisions is an area of law that is still developing.

If we are unable to maintain effective proprietary rights for our product candidates or any future product candidates, we may not be able to compete effectively in our proposed markets.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information, or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations, and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed, or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business, financial condition, or results of

operations. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our ability to develop, manufacture, market, and sell our product candidates and use our proprietary technology without infringing the patent rights of third parties. Numerous third-party U.S. and non-U.S. issued patents and pending applications exist in the area of microRNA. We are aware of U.S. and foreign patents and pending patent applications owned by third parties that cover therapeutic uses of microRNA replacements and inhibitors. From time to time, we may also monitor these patents and patent applications. We may in the future pursue available proceedings in the

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U.S. and foreign patent offices to challenge the validity of these patents and patent applications. In addition, or alternatively, we may consider whether to seek to negotiate a license of rights to technology covered by one or more of such patents and patent applications. If any patents or patent applications cover our product candidates or technologies, we may not be free to manufacture or market our product candidates, including cobomarsen, remlarsen, or MRG-110, as planned, absent such a license, which may not be available to us on commercially reasonable terms, or at all.

It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 remain confidential until patents issue and applications filed after that date that will not be filed outside the United States can elect to remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases, and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale, or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable, or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to specified limitations, be later amended in a manner that could cover our technologies, our product candidates, or the use of our product candidates.

There have been many lawsuits and other proceedings involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits in federal courts, and interferences, oppositions, inter partes reviews, post-grant reviews, and reexamination proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign-issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products, or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

We may not be successful in meeting our obligations under our existing license agreements necessary to maintain our product candidate licenses in effect. In addition, if required in order to commercialize our product candidates, we may be unsuccessful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we do not own, to develop and commercialize our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to maintain in effect these proprietary rights. Any termination of license agreements with third parties with respect to our product candidates would be expected to negatively impact our business prospects.

We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. Even if we are able to license or acquire third-party intellectual property rights that are necessary for our product candidates, there can be no assurance that they will be available on favorable terms.

We collaborate with U.S. and foreign academic institutions to identify product candidates, accelerate our research, and conduct development. Typically, these institutions have provided us with an option to negotiate an exclusive license to any of the institution’s rights in the patents or other intellectual property resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to

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pursue a program of interest to us.

If we are unable to successfully obtain and maintain rights to required third-party intellectual property, we may have to abandon development of that product candidate or pay additional amounts to the third party, and our business and financial condition could suffer.

The patent protection and patent prosecution for some of our product candidates is dependent on third parties.

While we normally seek and gain the right to fully prosecute the patents relating to our product candidates, there may be times when patents relating to our product candidates are controlled by our licensors. For instance, this is the case with our agreement with RICC, who is primarily responsible for the prosecution of patents and patent applications licensed to us under the applicable agreement. If RICC or any of our future licensors fail to appropriately and broadly prosecute and maintain patent protection for patents covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected, and we may not be able to prevent competitors from making, using, importing, and selling competing products. In addition, even where we now have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors in effect from actions prior to us assuming control over patent prosecution.

If we fail to comply with obligations in the agreements under which we license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license and supply agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing agreements impose, and we expect that future license agreements will impose, various diligence, milestone payments, royalties, purchasing, and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, our agreements may be subject to termination by the licensor, in which event we would not be able to develop, manufacture, or market products covered by the license or subject to supply commitments.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. If we or one of our licensing partners were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description, clarity, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to us from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with

litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade

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secrets of their former employers.

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we have written agreements and make every effort to ensure that our employees, consultants, and independent contractors do not use the proprietary information or intellectual property rights of others in their work for us, we may in the future be subject to any claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop our own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States.

These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries, particularly some developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Risks Related to Our Reliance on Third Parties

We rely on third parties to conduct our clinical trials, manufacture our product candidates, and perform other services. If these third parties do not successfully perform and comply with regulatory requirements, we may not be able to successfully complete clinical development, obtain regulatory approval, or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party CROs to conduct, monitor, and manage our ongoing clinical programs. We rely on these parties for execution of clinical trials, and we manage and control only some aspects of their activities. We remain responsible for ensuring that each of our trials is conducted in accordance with

the applicable protocol, legal, regulatory, and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with all applicable laws, regulations, and guidelines, including those required by the FDA and comparable foreign regulatory authorities for all of our product candidates in clinical development. If we or any of our CROs or vendors fail to comply with applicable laws, regulations, and guidelines, the results generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot be assured that our CROs and other vendors will meet these requirements, or that upon inspection by any regulatory authority, such regulatory authority will determine that efforts, including any of our clinical trials, comply with applicable requirements. Our failure to comply with these laws, regulations, and guidelines may require us to repeat clinical trials, which would be costly and delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with

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alternative CROs in a timely manner or do so on commercially reasonable terms. In addition, our CROs may not prioritize our clinical trials relative to those of other customers, and any turnover in personnel or delays in the allocation of CRO employees by the CRO may negatively affect our clinical trials. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, our clinical trials may be delayed or terminated, and we may not be able to meet our current plans with respect to our product candidates. CROs may also involve higher costs than anticipated, which could negatively affect our financial condition and operations.

In addition, we do not currently have, nor do we currently plan to establish, the capability to manufacture product candidates for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale without the use of third-party manufacturers. We plan to rely on third-party manufacturers and their responsibilities will include purchasing from third-party suppliers the materials necessary to produce our product candidates for our clinical trials and regulatory approval. There are expected to be a limited number of suppliers for the active ingredients and other materials that we expect to use to manufacture our product candidates, and we may not be able to identify alternative suppliers to prevent a possible disruption of the manufacture of our product candidates for our clinical trials, and, if approved, ultimately for commercial sale. Although we generally do not expect to begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the trial, any significant delay or discontinuity in the supply of a product candidate, or the active ingredient or other material components in the manufacture of the product candidate, could delay completion of our clinical trials and potential timing for regulatory approval of our product candidates, which would harm our business and results of operations.

We rely and expect to continue to rely on third parties to manufacture our clinical product supplies, and we intend to rely on third parties to produce and process our product candidates, if approved, and our commercialization of any of our product candidates could be stopped, delayed, or made less profitable if those third parties fail to obtain approval of government regulators, fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we currently plan to develop, the infrastructure or capability internally to manufacture our clinical supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on outside vendors to manufacture our clinical supplies of our product candidates and plan to continue relying on third parties to manufacture our product candidates on a commercial scale, if approved.

We do not yet have sufficient information to reliably estimate the cost of the commercial manufacturing of our product candidates and our current cost to manufacture our drug products may not be commercially feasible. Additionally, the actual cost to manufacture our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product.

In addition, our reliance on third-party manufacturers exposes us to the following additional risks:

- We may be unable to identify manufacturers on acceptable terms or at all.
- Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.
- Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
-

Our future third-party manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store, and distribute our products.

- Manufacturers are subject to ongoing periodic unannounced inspection by the FDA and some state agencies to ensure strict compliance with cGMPs and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.

- We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our product candidates.

- Our third-party manufacturers could breach or terminate their agreement with us.

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Each of these risks could delay our clinical trials, as well as the approval, if any, of our product candidates by the FDA, or the commercialization of our product candidates, or could result in higher costs, or could deprive us of potential product revenue. In addition, we rely on third parties to perform release testing on our product candidates prior to delivery to patients. If these tests are not appropriately conducted and test data are not reliable, patients could be put at risk of serious harm and could result in product liability suits.

The manufacture of medical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of medical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state, and foreign regulations. Furthermore, if contaminants are discovered in our supply of our product candidates or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot be assured that any stability or other issues relating to the manufacture of our product candidates will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidates to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

We may be unable to realize the potential benefits of any collaboration.

Even if we are successful in entering into a collaboration with respect to the development and/or commercialization of one or more product candidates, there is no guarantee that the collaboration will be successful. Collaborations may pose a number of risks, including:

- collaborators often have significant discretion in determining the efforts and resources that they will apply to the collaboration and may not commit sufficient resources to the development, marketing, or commercialization of the product or products that are subject to the collaboration;
- collaborators may not perform their obligations as expected;
- any such collaboration may significantly limit our share of potential future profits from the associated program and may require us to relinquish potentially valuable rights to our current product candidates, potential products, proprietary technologies, or grant licenses on terms that are not favorable to us;
- collaborators may cease to devote resources to the development or commercialization of our product candidates if the collaborators view our product candidates as competitive with their own products or product candidates;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation, or the course of development, might cause delays or termination of the development or commercialization of product candidates, and might result in legal proceedings, which would be time consuming, distracting, and expensive;
- collaborators may be impacted by changes in their strategic focus or available funding, or business combinations involving them, which could cause them to divert resources away from the collaboration;

- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the collaborations may not result in us achieving revenues to justify such transactions; and
- collaborations may be terminated and, if terminated, may result in a need for us to raise additional capital to pursue further development or commercialization of the applicable product candidate.

As a result, a collaboration may not result in the successful development or commercialization of our product candidates.

For instance, in October 2011, we entered into the Servier Collaboration Agreement with Servier for the research,

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development, and commercialization of RNA-targeting therapeutics in cardiovascular disease, which was subsequently amended. Under the Servier Collaboration Agreement, we have granted Servier an exclusive license to research, develop, and commercialize RNA-targeting therapeutics for one named target in the cardiovascular field and the right to obtain such an exclusive license for one additional target through September 2019. Servier's rights to a given target are limited to therapeutics in the cardiovascular field in their territory, which is worldwide except for the United States and Japan. We retain all rights for the named target in the United States and Japan in the cardiovascular field and worldwide for any products or product candidates outside of the cardiovascular field. We cannot guarantee that any product candidate will ever be successfully commercialized under the Servier Collaboration Agreement. If no product candidate subject to the Servier Collaboration Agreement is successfully commercialized, we may never receive additional milestone or any royalty payments under the Servier Collaboration Agreement. Also, due to restrictions contained in the Servier Collaboration Agreement, we may not be able to effectively develop, market, or commercialize any such product candidate in the United States and Japan.

We enter into various contracts in the normal course of our business in which we indemnify the other party to the contract. In the event we have to perform under these indemnification provisions, we could have a material adverse effect on our business, financial condition, and results of operations.

In the normal course of business, we periodically enter into academic, commercial, service, collaboration, licensing, consulting, and other agreements that contain indemnification provisions. With respect to our academic and other research agreements, we typically indemnify the institution and related parties from losses arising from claims relating to the products, processes, or services made, used, sold, or performed pursuant to the agreements for which we have secured licenses, and from claims arising from our or our sublicensees' exercise of rights under the agreement. With respect to our collaboration agreements, we indemnify our collaborators from any third-party product liability claims that could result from the production, use, or consumption of the product, as well as for alleged infringements of any patent or other intellectual property right by a third party. With respect to consultants, we indemnify them from claims arising from the good faith performance of their services.

Should our obligation under an indemnification provision exceed applicable insurance coverage or if we were denied insurance coverage, our business, financial condition, and results of operations could be adversely affected. Similarly, if we are relying on a collaborator to indemnify us and the collaborator is denied insurance coverage or the indemnification obligation exceeds the applicable insurance coverage, and if the collaborator does not have other assets available to indemnify us, our business, financial condition, and results of operations could be adversely affected.

Risks Related to Commercialization of Our Product Candidates

We currently have limited marketing and sales experience. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

Although some of our employees may have been employed at companies that have launched pharmaceutical products in the past, we have no experience selling and marketing our product candidates and we currently have no marketing or sales organization. To successfully commercialize any products that may result from our development programs, we will need to find one or more collaborators to commercialize our products or invest in and develop these capabilities, either on our own or with others, which would be expensive, difficult, and time consuming. Any failure or delay in entering into agreements with third parties to market or sell our product candidates or in the timely development of our internal commercialization capabilities could adversely impact the potential for the launch and success of our products.

If commercialization collaborators do not commit sufficient resources to commercialize our future products and we are unable to develop the necessary marketing and sales capabilities on our own, we will be unable to generate sufficient product revenue to sustain or grow our business. We may be competing with companies that currently have extensive and well-funded marketing and sales operations, particularly in the markets our product candidates are intended to address. Without appropriate capabilities, whether directly or through third-party collaborators, we may be unable to compete successfully against these more established companies.

We may attempt to form collaborations in the future with respect to our product candidates, but we may not be able to do so, which may cause us to alter our development and commercialization plans.

We may attempt to form strategic collaborations, create joint ventures, or enter into licensing arrangements with third parties with respect to our programs that we believe will complement or augment our existing business. We may face significant competition in seeking appropriate strategic collaborators, and the negotiation process to secure appropriate terms is time

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consuming and complex. We may not be successful in our efforts to establish such a strategic collaboration for any product candidates and programs on terms that are acceptable to us, or at all. This may be because our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort, our research and development pipeline may be viewed as insufficient, the competitive or intellectual property landscape may be viewed as too intense or risky, and/or third parties may not view our product candidates and programs as having sufficient potential for commercialization, including the likelihood of an adequate safety and efficacy profile.

Any delays in identifying suitable collaborators and entering into agreements to develop and/or commercialize our product candidates could delay the development or commercialization of our product candidates, which may reduce their competitiveness even if they reach the market. Absent a strategic collaborator, we would need to undertake development and/or commercialization activities at our own expense. If we elect to fund and undertake development and/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 are unable to do so, we may not be able to develop our product candidates or bring them to market and our business may be materially and adversely affected.

If the market opportunities for our product candidates are smaller than we believe they are, we may not meet our revenue expectations and, assuming approval of a product candidate, our business may suffer. Because the patient populations in the market for our product candidates may be small, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

Given the small number of patients who have the diseases that we are targeting, our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our product candidates. For instance, the lead indication of cobomarsen is MF. The estimated prevalence of MF is 16,000 to 20,000 cases in the United States, only a subset of which may benefit from treatment with cobomarsen. Our projections of both the number of people who have this disease, as well as the subset of people with this disease who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. Additionally, while we believe that the data in our Phase 1 clinical trials for cobomarsen and remlarsen are supportive of application to other indications, there can be no assurance that our clinical trials in those indications will support efficacy of our product candidates in such expanded indications. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our business, financial condition, results of operations and prospects.

We face substantial competition and our competitors may discover, develop, or commercialize products faster or more successfully than us.

The development and commercialization of new drug products is highly competitive. We face competition from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, universities, and other research institutions worldwide with respect to cobomarsen, remlarsen, MRG-110, and the other product candidates that we may seek to develop or commercialize in the future. We are aware that the following companies have therapeutics marketed or in development for CTCL: Argenx, Bristol-Myers Squibb Company, Celgene Corporation, Helsinn Group, innate Pharma, Kyowa Hakko Kirin, Merck & Co., Inc., Mylan Pharmaceuticals Inc., Novartis International AG, Spectrum Pharmaceuticals, Inc., Seattle Genetics, Inc., Takeda Pharmaceutical Company Ltd, and Valeant Pharmaceuticals International, Inc. We are also aware that the several companies have marketed therapeutics for pulmonary fibrosis, including Boehringer Ingelheim GmbH and F. Hoffmann-La Roche Ltd. Our competitors may succeed in developing, acquiring, or licensing technologies and drug products that are more effective or less costly than cobomarsen, remlarsen, MRG-110, or any other product candidates that we are currently developing or that we

may develop, which could render our product candidates obsolete and noncompetitive.

In addition to the competition we face from alternative therapies for the diseases we intend to target with our product candidates, we are also aware of several companies that are also working specifically to develop microRNA-targeted therapeutics, including Regulus Therapeutics, Inc., and InteRNA Technologies, B.V. Further, there are several companies working to develop other types of oligonucleotide therapeutic products, including Ionis Pharmaceuticals, Inc., Alnylam Pharmaceuticals, Inc., Arrowhead Pharmaceuticals, Inc., Dicerna Pharmaceuticals, Inc., STELLAS Life Sciences Group, Inc., Silence Therapeutics AG, and Translate Bio, Inc. Many of our competitors have substantially greater financial, technical, and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Third-party payors, including governmental and private insurers, may also encourage the use of generic products. For example, if cobomarsen, remlarsen, or MRG-110 is approved, it may be priced at a significant premium over

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other competitive products. This may make it difficult for cobomarsen, remlarsen, MRG-110, or any other future products to compete with these products.

If our competitors obtain marketing approval from the FDA or comparable foreign regulatory authorities for their product candidates more rapidly than us, it could result in our competitors establishing a strong market position before we are able to enter the market.

Many of our competitors have materially greater name recognition and financial, manufacturing, marketing, research, and drug development resources than we do. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Large pharmaceutical companies in particular have extensive expertise in preclinical and clinical testing and in obtaining regulatory approvals for drugs. In addition, academic institutions, government agencies, and other public and private organizations conducting research may seek patent protection with respect to potentially competitive products or technologies. These organizations may also establish exclusive collaborative or licensing relationships with our competitors. Failure of cobomarsen, remlarsen, MRG-110, or other product candidates to effectively compete against established treatment options or in the future with new products currently in development would harm our business, financial condition, results of operations, and prospects.

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

Even with the approvals from the FDA and comparable foreign regulatory authorities, the commercial success of our products will depend in part on the healthcare providers, patients, and third-party payors accepting our product candidates as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, and third-party payors. The degree of market acceptance of any of our products will depend on a number of factors, including but not limited to:

- the efficacy of the product as demonstrated in clinical trials and potential advantages over competing treatments;
- the prevalence and severity of the disease and any side effects;
- the clinical indications for which approval is granted, including any limitations or warnings contained in a product's approved labeling;
- the convenience and ease of administration;
- the cost of treatment;
- the willingness of the patients and physicians to accept these therapies;
- the perceived ratio of risk and benefit of these therapies by physicians and the willingness of physicians to recommend these therapies to patients based on such risks and benefits;
- the marketing, sales, and distribution support for the product;
- the publicity concerning our products or competing products and treatments; and
- the pricing and availability of third-party payor coverage and adequate reimbursement.

Even if a product displays a favorable efficacy and safety profile upon approval, market acceptance of the product remains uncertain. Efforts to educate the medical community and third-party payors on the benefits of the products may require significant investment and resources and may never be successful. If our products fail to achieve an adequate level of acceptance by physicians, patients, third-party payors, and other healthcare providers, we will not be able to generate sufficient revenue to become or remain profitable.

We may not be successful in any efforts to identify, license, discover, develop, or commercialize additional product candidates.

Although a substantial amount of our effort will focus on the continued clinical testing, potential approval, and commercialization of our existing product candidates, the success of our business is also expected to depend in part upon our

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ability to identify, license, discover, develop, or commercialize additional product candidates. Research programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. Our research programs or licensing efforts may fail to yield additional product candidates for clinical development and commercialization for a number of reasons, including but not limited to the following:

- our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates;
- we may not be able or willing to assemble sufficient resources to acquire or discover additional product candidates;
- our product candidates may not succeed in preclinical or clinical testing;
- our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the market for a product candidate may change during our program so that such a product may become unreasonable to continue to develop;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, license, discover, develop, or commercialize additional product candidates, which would have a material adverse effect on our business, financial condition, or results of operations and could potentially cause us to cease operations.

Failure to obtain or maintain adequate reimbursement or insurance coverage for our products, if any, could limit our ability to market those products and decrease our ability to generate revenue.

The pricing, as well as the coverage, and reimbursement of our approved products, if any, must be sufficient to support our commercial efforts and other development programs, and the availability of coverage and adequacy of reimbursement by third-party payors, including government healthcare programs, health maintenance organizations, private insurers, and other healthcare management organizations, are essential for most patients to be able to afford expensive treatments. Sales of our approved products, if any, will depend substantially, both domestically and abroad, on the extent to which the costs of our approved products, if any, will be paid for or reimbursed by third-party payors. If coverage and reimbursement are not available, or are available only in limited amounts, we may have to subsidize or provide products for free, or we may not be able to successfully commercialize our products.

In addition, there is significant uncertainty related to the insurance coverage and reimbursement for newly-approved products. In the United States, the principal decisions about coverage and reimbursement for new drugs are typically made by CMS, which is an agency within the U.S. Department of Health and Human Services that decides whether

and to what extent a new drug will be covered and reimbursed under Medicare. Private third-party payors tend to follow the coverage and reimbursement policies established by CMS to a substantial degree, but also have their own methods and approval process apart from Medicare determinations. It is difficult to predict what CMS will decide with respect to reimbursement for novel product candidates such as our and what reimbursement codes our product candidates may receive if approved.

Outside the United States, international operations are generally subject to extensive governmental price controls and other price-restrictive regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of products. In many countries, the prices of products are subject to varying price control mechanisms as part of national health systems. Price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products, if any. Accordingly, in markets outside the United States, the potential revenue may be insufficient to generate commercially reasonable revenue and profits.

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Moreover, increasing efforts by third-party payors in the United States and abroad to limit or reduce healthcare costs may result in restrictions on coverage and the level of reimbursement for new products and, as a result, they may not cover or provide adequate payment for our products. Further, 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 and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. For example, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers.

We expect to experience pricing pressures in connection with products due to the increasing trend toward managed healthcare, including the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs, has increased and is expected to continue to increase in the future. As a result, profitability of our products, if any, may be more difficult to achieve even if they receive regulatory approval.

Risks Related to Our Business Operations

Our future success depends in part on our ability to retain our president and chief executive officer and to attract, retain, and motivate other qualified personnel.

We are highly dependent on William S. Marshall, Ph.D., our president and chief executive officer, the loss of whose services may adversely impact the achievement of our objectives. Dr. Marshall could leave our employment at any time, as he is an "at will" employee. Recruiting and retaining other qualified employees, consultants, and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of highly qualified personnel in our industry, which is likely to continue. Additionally, this shortage of highly qualified personnel is particularly acute in the area where we are located. As a result, competition for personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in development and commercialization of our product candidates may make it more challenging to recruit and retain qualified personnel. The inability to recruit and retain qualified personnel, or the loss of the services of Dr. Marshall, may impede the progress of our research, development, and commercialization objectives and would negatively impact our ability to succeed in our product development strategy.

We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As of December 31, 2018, we had 77 full-time employees. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal, and other resources. Our management may need to divert a disproportionate amount of our attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be

reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Failure in our information technology and storage systems could significantly disrupt the operation of our business.

Our ability to execute our business plan and maintain operations depends on the continued and uninterrupted performance of our information technology, or IT, systems. IT systems are vulnerable to risks and damages from a variety of sources, including telecommunications or network failures, malicious human acts, and natural disasters. Moreover, despite network security and back-up measures, some of our and our vendors' servers are potentially vulnerable to physical or electronic break-ins, including cyber-attacks, computer viruses, and similar disruptive problems. These events could lead to the unauthorized access, disclosure, and use of non-public information. The techniques used by criminal elements to attack

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computer systems are sophisticated, change frequently, and may originate from less regulated and remote areas of the world. As a result, we may not be able to address these techniques proactively or implement adequate preventative measures. If our computer systems are compromised, we could be subject to fines, damages, litigation, and enforcement actions, and we could lose trade secrets, the occurrence of which could harm our business. Despite precautionary measures to prevent unanticipated problems that could affect our IT systems, sustained or repeated system failures that interrupt our ability to generate and maintain data could adversely affect our ability to operate our business.

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

Our net operating loss, or NOL, carryforwards could expire unused and be unavailable to offset future income tax liabilities. because of their limited duration or because of restrictions under U.S. tax law. Our NOLs generated in tax years beginning on or prior to December 31, 2017 are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under H.R. 1, “An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018”, informally titled the Tax Act, our federal NOLs generated in tax years beginning after December 31, 2017 may be carried forward indefinitely, but the deductibility of such federal NOLs is limited. It is uncertain if and to what extent various states will conform to the Tax Act.

In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, 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 NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. Our most recent analysis of possible ownership changes was completed for certain tax periods ending through the date of the Merger. The Merger resulted in an ownership change for us and, accordingly, our NOL carryforwards and certain other tax attributes are subject to limitation. It is possible that we have undergone additional ownership changes before and after the Merger, and additional ownership changes in the future could result in additional limitations on our NOL carryforwards.

Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our NOL carryforwards and certain other tax attributes, which could have a material adverse effect on cash flow and results of operations.

New or future changes to tax laws could materially adversely affect our company.

The Tax Act significantly revises the Code. The Tax Act, among other things, reduces the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limits the tax deduction for interest expense to 30% of adjusted taxable income (except for certain small businesses), limits the deduction for NOLs carried forward from taxable years beginning after December 31, 2017 to 80% of current year taxable income, eliminates NOL carrybacks, imposes a one-time tax on offshore earnings at reduced rates regardless of whether they are repatriated, eliminates U.S. tax on foreign earnings (subject to certain important exceptions), allows immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifies or repeals many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the Tax Act 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 Tax Act. The impact of the Tax Act or any future tax laws 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 effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

We are subject to taxation in numerous U.S. states and territories and non-U.S. jurisdictions. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors including passage of the newly enacted federal income tax law, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes, and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

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Risks Related to Ownership of our Common Stock

The market price of our common stock is expected to be volatile, and the market price of our common stock may drop in the future.

The market price of our common stock following the Merger has been, and may continue to be, subject to significant fluctuations. Market prices for securities of early-stage pharmaceutical, biotechnology, and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- our ability to obtain regulatory approvals for cobomarsen, remlarsen, MRG-110, or other product candidates, and delays or failures to obtain such approvals;
- failure of any of our product candidates, if approved, to achieve commercial success;
- failure to maintain our existing third-party license and supply agreements;
- changes in laws or regulations applicable to our product candidates;
- any inability to obtain adequate supply of our product candidates or the inability to do so at acceptable prices;
- adverse regulatory authority decisions;
- introduction of new products, services, or technologies by our competitors;
- failure to meet or exceed financial and development projections we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators, and the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures, or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our business and stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions;
- sales of our common stock by us or our stockholders in the future;

- trading volume of our common stock;
- announcements by commercial partners or competitors of new commercial products, clinical progress or the lack thereof, significant contracts, commercial relationships, or capital commitments;
- adverse publicity relating to microRNA-targeted therapeutics generally, including with respect to other products and potential products in such markets;
- the introduction of technological innovations or new therapies that compete with our potential products;

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- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the capital markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

Additionally, a decrease in our stock price may cause our common stock to no longer satisfy the continued listing standards of The Nasdaq Capital Market. If we are not able to maintain the requirements for listing on The Nasdaq Capital Market, we could be delisted, which could have a materially adverse effect on our ability to raise additional funds as well as the price and liquidity of our common stock.

We incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

As a result of the Merger, we incur significant legal, accounting, and other expenses that Private Miragen did not incur as a private company, including costs associated with public company reporting requirements. We also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as new rules implemented by the SEC and The Nasdaq Stock Market LLC, or Nasdaq. These rules and regulations are expected to increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, our management team consists of the executive officers of Private Miragen prior to the Merger, some of whom have not previously managed and operated a public company. These executive officers and other personnel will need to devote substantial time to gaining expertise regarding operations as a public company and compliance with applicable laws and regulations. These rules and regulations may also make it difficult and expensive for us to obtain directors' and officers' liability insurance. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as our executive officers, which may adversely affect investor confidence and could cause our business or stock price to suffer.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our certificate of incorporation and bylaws may delay or prevent an acquisition or a change in management. These provisions include a prohibition on actions by written consent of our stockholders and the ability of our board of directors to issue preferred stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which prohibits stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Our bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or other employees.

Our bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty owed by any of our directors, officers, or other employees to us or our stockholders, any action asserting a claim against us arising pursuant to any provisions of the Delaware General Corporation Law, our certificate of incorporation or our bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage such lawsuits against our and our directors, officers, and other employees. If a court were to find the choice of forum provision contained in the bylaws to be inapplicable or unenforceable in an action, we may incur

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additional costs associated with resolving such action in other jurisdictions.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

The current expectation is that we will retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future.

Historically, there has not been an active trading market for our common stock, and we cannot guarantee an active market for our common stock will be sustained in the future. As a result, our stockholders may not be able to resell their shares of common stock for a profit, if at all.

Prior to the Merger, there had been no public market for Private Miragen's common stock. An active trading market for our shares of common stock has yet to develop, and even if an active market for our common stock were to develop, it may not be sustained. If an active market for our common stock is not sustained, it may be difficult for our stockholders to sell their shares at an attractive price or at all.

Future sales of shares by existing stockholders could cause our stock price to decline.

If our stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after legal restrictions on resale lapse, the trading price of our common stock could decline. In addition, shares of common stock that are subject to our outstanding options will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements and Rules 144 and 701 under the Securities Act of 1933, as amended.

Our principal stockholders own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Our directors, officers, 5% stockholders, and their affiliates currently beneficially own a substantial portion of our outstanding voting stock. Therefore, these stockholders have the ability and may continue to have the ability to influence us through this ownership position. These stockholders may be able to determine some or all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

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. Equity research analysts may elect not to provide research coverage of our common stock and such lack of research coverage may adversely affect the market price of our common stock. In the event we do 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 common 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 us or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

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 Exchange Act, the Sarbanes-Oxley Act, and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must 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 controls over financial reporting in our annual report filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. As a private company, Private Miragen had never been required to test its internal controls within a specified period. This requires that we incur substantial professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. We may experience difficulty in meeting these reporting requirements in a timely manner for each period.

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We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. 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.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our common stock could decline, and it could be subject to sanctions or investigations by Nasdaq, the SEC, or other regulatory authorities.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We lease 27,128 square feet of office and laboratory space in Boulder, Colorado under a lease that expires in August 2020, subject to two three-year renewal options prior to the expiration, and that includes rent escalation clauses through the lease term. We believe that this space is suitable for our current needs.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may be involved in legal proceedings in the ordinary course of business. We are currently not a party to any legal proceedings that we believe would have a material adverse effect on our business, financial condition, or results of operations.

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

On February 13, 2017, Signal and Private Miragen completed the Merger. Following the Merger, Private Miragen merged with and into Signal, with Signal as the surviving corporation. In connection with the Mergers, we changed the name of the combined company to Miragen Therapeutics, Inc. and changed the trading symbol for our common stock on The Nasdaq Capital Market to "MGEN."

Our common stock originally began trading on The Nasdaq Capital Market on June 17, 2014 under the trading symbol "SGNL." Prior to June 17, 2014, there was no public market for our common stock.

Holders

As of March 1, 2019, we had 17 registered holders of record of our common stock. A substantially greater number of holders of our common stock are "street name" or beneficial holders, whose shares of record are held by banks, brokers, other financial institutions, and registered clearing agencies.

Dividend Policy

We historically have not, and do not anticipate in the future, paying dividends on our common stock. We currently intend to retain all of our future earnings, as applicable, to finance the growth and development of our business. We are not subject to any legal restrictions respecting the payment of dividends, except that we may not pay dividends if the payment would render us insolvent. We are subject to certain dividend-related limitations under our loan and security agreement with Silicon Valley Bank. Subject to these limitations, any future determination as to the payment of cash dividends on our common stock will be at our board of directors' discretion and will depend on our financial condition, operating results, capital requirements and other factors that our board of directors considers to be relevant.

ITEM 6. SELECTED FINANCIAL DATA

The following selected consolidated financial data is derived from our audited consolidated financial statements and should be read in conjunction with the consolidated financial statements, the notes to such statements and "Management's Discussion and Analysis of Financial Condition and Results of Operations" included elsewhere in this Annual Report. Historical results are not necessarily indicative of the results to be expected in the future.

	Year Ended December 31,	
	2018	2017
	(in thousands, except share and per share data)	
Revenue:		
Collaboration revenue	$7,373	$3,097
Grant revenue	1,013	906
Total revenue	8,386	4,003
Operating expenses:		
Research and development	30,421	19,623
General and administrative	11,049	10,912
Total operating expenses	41,470	30,535
Loss from operations	(33,084)	(26,532)
Other income (expense):		
Interest and other income	1,254	403
Interest and other expense	(873)	(383)
Net loss	(32,703)	(26,512)
Change in unrealized loss on investments	(3)	—
Comprehensive loss	$(32,706)	$(26,512)
Net loss	$(32,703)	$(26,512)
Accretion of redeemable convertible preferred stock to redemption value	—	(5)
Net loss available to common stockholders	$(32,703)	$(26,517)
Net loss per share, basic and diluted	$(1.10)	$(1.38)
Weighted-average shares used to compute basic and diluted net loss per share	29,600,332	19,244,605

	December 31,	
	2018	2017
	(in thousands)	
Cash and cash equivalents	$32,606	$47,441
Short-term investments	$29,875	$—
Total assets	$66,147	$52,481
Note payable, inclusive of current portion	$10,298	$9,922
Total liabilities	$14,803	$13,971
Total stockholders' equity	$51,344	$38,510

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

The following discussion and analysis should be read together with our consolidated financial statements and the related notes thereto appearing elsewhere in this Annual Report. This discussion and other parts of this report contain forward-looking statements reflecting our current expectations that involve risks and uncertainties, such as our plans, objectives, expectations, intentions, and beliefs. See "Forward-Looking Statements" for a discussion of the uncertainties, risks, and assumptions associated with these statements. Actual results and the timing of events could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the section entitled "Risk Factors" included elsewhere in this Annual Report.

All references to 2018 and 2017 refer to calendar years ended December 31, 2018 and 2017, respectively.

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Overview

We are a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in certain diseases where there is a high unmet medical need. microRNAs are short RNA molecules, or oligonucleotides, that regulate gene expression and play vital roles in influencing the pathways responsible for many disease processes. We have three product candidates, cobomarsen, remlarsen and MRG-110, in clinical development. We believe each has the potential to treat multiple indications. We are independently developing cobomarsen and remlarsen and are developing MRG-110 in collaboration with Servier.

Cobomarsen is an inhibitor of miR-155, which is a microRNA that is found at abnormally high levels in malignant cells of several blood cancers. Remlarsen is a replacement for miR-29, a microRNA that is found at abnormally low levels in a number of pathological fibrotic conditions. MRG-110 is an inhibitor of miR-92, a microRNA expressed in endothelial cells. MRG-110 is being developed for the treatment of heart failure, as well as surgical incisions in high risk populations, severe lacerations, and severe burns. We retain all commercial rights to MRG-110 in the United States and Japan, and Servier has commercial rights to MRG-110 for cardiovascular indications in the rest of the world.

In addition to our clinical-stage programs, we continue to develop a pipeline of preclinical product candidates. The goal of our translational medicine strategy is to progress rapidly to first-in-human trials once we have adequately established the pharmacokinetics (the movement of a drug into, through, and out of the body), pharmacodynamics (the effect and mechanism of action of a drug), safety, and manufacturability of the product candidate in preclinical studies.

We believe our experience in microRNA biology and chemistry, drug discovery, bioinformatics, translational medicine, and drug development allows us to identify and develop mircroRNA-targeted drugs that are designed to regulate gene pathways to return diseased tissues to a healthy state. We believe that our drug discovery and development strategy will enable us to progress our product candidates from preclinical discovery to confirmation of mechanism of action in humans quickly and efficiently. The elements of this strategy include identification of biomarkers that may predict clinical benefit and monitoring outcomes in early-stage clinical trials to help guide later clinical development.

Financial Operations Overview

Revenue

Our revenue consists primarily of upfront payments for licenses, milestone payments, and payments for other research and development services earned under our strategic alliance and collaboration agreement. We also recognize revenue for amounts received or receivable under certain grants we have been awarded.

In the future, we may generate revenue from a combination of license fees and other upfront payments, payments for research and development services, milestone payments, product sales, and royalties in connection with strategic alliances. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing of our achievement of preclinical, clinical, regulatory, and commercialization milestones, the timing and amount of payments relating to such milestones, and the extent to which any of our products are approved and successfully commercialized by us or our strategic alliance partners. If our strategic alliance partners do not elect or otherwise agree to fund our development costs pursuant to our strategic alliance agreements, or we or our strategic alliance partners fail to develop product candidates in a timely manner or to obtain regulatory approval for them, then our ability to generate future revenues, and our results of operations and financial position would be adversely affected.

Research and development expenses

Research and development expenses consist of costs incurred for the research and development of our therapeutic programs and product candidates, which include:

- employee-related expenses, including salaries, benefits, travel, and share-based compensation expense;
- expenses incurred under agreements with CROs, investigative sites that conduct our clinical trials, and other clinical trial-related vendors, consultants, and our scientific advisors;
- the costs of acquiring, developing, and manufacturing clinical trial materials, including costs incurred under agreements with contract manufacturing organizations, or CMOs;

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- costs associated with non-clinical activities and regulatory operations;

- license fees and milestone payments related to the acquisition and retention of certain licensed technology and intellectual property rights; and

- facilities, depreciation, market research, and other expenses, which include expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We make non-refundable advance payments for goods and services that will be used in future research and development activities. These payments are recorded as expense in the period in which we receive or take ownership of the goods or when the services are performed.

We record upfront and milestone payments to acquire and retain contractual rights to in-licensed technology and intellectual property rights as research and development expenses when incurred if there is uncertainty in our receiving future economic benefit from the acquired contractual rights. We consider future economic benefits from acquired contractual rights to licensed technology to be uncertain until such a drug candidate is approved by the FDA or when other significant risk factors are abated.

We expect our research and development expenses to increase for the foreseeable future as we continue to conduct our ongoing clinical trials, initiate new clinical trials, and advance our preclinical research programs. The process of conducting clinical trials and preclinical studies necessary to obtain regulatory approval is costly and time consuming. We, or our strategic alliance partner, may never succeed in achieving marketing approval for any of our product candidates. The probability of success for each product candidate may be affected by numerous factors, including clinical data, preclinical data, competition, manufacturability, and commercial viability of our product candidates.

Successful development of future product candidates is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each future product candidate and are difficult to predict. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to our ability to maintain or enter into new strategic alliances with respect to each program or potential product candidate, the scientific and clinical success of each future product candidate, and ongoing assessments as to each future product candidate’s commercial potential. We will need to raise additional capital and may seek additional strategic alliances in the future in order to advance our various programs.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related benefits, including share-based compensation, related to our finance, accounting, human resources, legal, business development, and other support functions, professional fees for auditing, tax, and legal services, as well as insurance, board of director compensation, and other administrative expenses.

Other income, net

Other income, net consists primarily of interest income and expense, and various income or expense items of a non-recurring nature. We earn interest income from interest-bearing accounts, money market funds, and short-term investments. Interest expense is comprised of interest incurred under our note payable.

Critical Accounting Policies and Estimates

This discussion and analysis of financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policy discussed below is critical to understanding our historical and future performance, as this policy relates to the more significant areas involving our judgments and estimates.

Clinical Trial and Preclinical Study Accruals

We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on

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certain facts and circumstances at that time. Our accrued expenses for preclinical studies and clinical trials are based on estimates of costs incurred for services provided by external service providers and for other trial-related activities. The timing and amount of expenses we incur though our external service providers depend on a number of factors, such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, we obtain information from various sources and estimate the level of effort or expense allocated to each period. Adjustments to our research and development expenses may be necessary in future periods as our estimates change.

Results of Operations

Comparison of the Year Ended December 31, 2018 and 2017

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Revenue	$8,386	$4,003
Research and development expenses	(30,421)	(19,623)
General and administrative expenses	(11,049)	(10,912)
Other income, net	381	20
Net loss	$(32,703)	$(26,512)

Revenue

Revenue increased to $8.4 million during the year ended December 31, 2018, from $4.0 million during the year ended December 31, 2017. The increase was due primarily to a €3.0 million (or $3.7 million) milestone payment earned and received under the Servier Collaboration Agreement during the year ended December 31, 2018, as well as a $0.6 million increase in research and development and intellectual property activities reimbursable to us by Servier under the Servier Collaboration Agreement.

Research and Development Expenses

Research and development expenses were $30.4 million during the year ended December 31, 2018, compared to $19.6 million during the year ended December 31, 2017. The increase in research and development expense of $10.8 million was driven primarily by:

- increased clinical development and related manufacturing expenses of $7.4 million, primarily related to expenses incurred in connection with additional clinical trial costs, including manufacturing costs, for the Phase 2 SOLAR clinical trial of cobomarsen and the initiation of a Phase 1 clinical trial of MRG-110 during of 2018;

- increased personnel-related costs of $2.4 million, including share-based compensation, due primarily to the growth of our research and development team; and

- increased technology license fees of $0.9 million primarily related to a milestone payment due to one of our licensors for the initiation of our first clinical trial of a compound targeting miR-92 during 2018.

General and Administrative Expenses

General and administrative expenses were $11.0 million during the year ended December 31, 2018, compared to $10.9 million during the year ended December 31, 2017. During 2018, personnel-related and professional

administrative costs increased by $1.0 million, due primarily to the growth of our general and administrative team and increased share-based compensation charges. This increase was offset by a decrease in legal expenses of $0.9 million, primarily related to non-recurring expenses related to the Merger that occurred in 2017.

Liquidity and Capital Resources

We have no products approved for commercial sale and have not generated any revenue from product sales. We have funded our operations to date principally through proceeds from convertible debt and equity financings.

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In August 2018, we and LLS entered into the LLS Stock Purchase Agreement for the sale of up to $5.0 million of shares of our common stock to LLS in a private placement, or the Offering. Under the terms of the LLS Stock Purchase Agreement, we expect to raise up to approximately $5.0 million in gross proceeds by selling shares of our common stock to LLS in up to five separate closings. At the initial closing in August 2018, we issued 150,987 shares of our common stock at a price per share equal to $6.62, which resulted in net proceeds of approximately $0.9 million after expenses incurred in connection with the Offering. The price per share of our common stock to be sold in any subsequent closing will be equal to the average of the volume weighted-average prices of a share of our common stock on the Nasdaq Capital Market for the three trading days beginning with the first trading day after the date of achievement of the relevant milestone for each such closing. Each closing is subject to our achievement of specified operational milestones under the LLS Stock Purchase Agreement and other customary closing conditions, provided, however, that each such closing must be completed prior to December 31, 2021.

In February 2018, we entered into the Underwriting Agreement, with the Underwriters, relating to a public offering of our common stock. In this offering, we sold 7,414,996 shares of common stock at a price of $5.50 per share, which resulted in net proceeds of approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses.

In March 2017, we entered into the ATM 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 $50.0 million through Cowen as our sales agent. Through March 14, 2019, we sold an aggregate of 1,260,975 shares of our common stock for aggregate net proceeds of approximately $10.3 million, after deducting commissions to Cowen as sales agent and initial expenses for executing the “at the market offering”.

Since our inception and through December 31, 2018, we have generated cumulative losses of $126.3 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We will continue to require substantial additional capital to continue our clinical development and potential commercialization activities. Accordingly, we will need substantial additional capital to continue to fund our operations. The amount and timing of future funding requirements will depend on many factors, including the pace and results of our clinical development efforts, continued performance under the Servier Collaboration Agreement, securing additional partnerships and collaborations, and issuing debt or other financing vehicles. Our ability to secure capital is dependent upon a number of factors, including success in developing our technology and product candidates. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates. Changing circumstances may cause us to consume capital significantly faster or slower than we currently anticipate. If we are unable to acquire additional capital or resources, we will be required to modify our operational plans to complete future milestones. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available financial resources sooner than we currently anticipate. We may be forced to reduce our operating expenses and raise additional funds to meet our working capital needs, principally through the additional sales of our securities or debt financings or entering into strategic collaborations.

We expect to incur significant expenses and increased operating losses for at least the next several years as we continue the clinical development of, and seek regulatory approval for, our product candidates and add personnel necessary to operate as a public company with an advanced clinical candidate pipeline of product candidates. In addition, operating as a publicly-traded company involves the hiring of additional financial and other personnel, upgrading financial information systems, and incurring costs associated with operating as a public company. We expect that our operating losses will fluctuate significantly from quarter to quarter and year to year due to timing of

clinical development programs and efforts to achieve regulatory approval.

If we raise additional funds through the issuance of debt, the obligations related to such debt could be senior to rights of holders of our capital stock and could contain covenants that may restrict our operations. Should additional capital not be available to us in the near term, or not be available on acceptable terms, we may be unable to realize value from our assets and discharge our liabilities in the normal course of business, which may, among other alternatives, cause us to further delay, substantially reduce, or discontinue operational activities to conserve our cash resources.

As of December 31, 2018, we had cash and cash equivalents of $32.6 million and short-term investments of $29.9 million. We believe our current resources will be sufficient to fund our operations in the normal course of business and allow us to meet our liquidity needs through the first quarter of 2020.

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Summarized cash flows for the year ended December 31, 2018 and 2017 are as follows:

	Year Ended December 31,		
	2018	2017	Change
	(in thousands)		
Net cash used in operating activities	$(26,844)	$(28,167)	$1,323
Net cash provided by (used in) investing activities	(29,907)	1,034	(30,941)
Net cash provided by financing activities	41,916	52,470	(10,554)
Net increase (decrease) in cash and cash equivalents	$(14,835)	$25,337	$(40,172)

Operating Activities

Net cash used in operating activities was $26.8 million for the year ended December 31, 2018, compared to $28.2 million for the year ended December 31, 2017. The decrease was primarily the result of a $6.4 million decrease in payments of associated current liabilities, receipts associated with accounts receivable, and payments associated with prepaid expenses and other assets during the year ended December 31, 2018, as well as a $6.2 million increase in net loss, offset by a $1.3 million increase in share-based compensation.

Investing Activities

Net cash used in investing activities was $29.9 million during the year ended December 31, 2018 compared to net cash provided by investing activities of $1.0 million during the year ended December 31, 2017. The change in cash flow from investing activities was driven primarily by $62.5 million of cash and cash equivalents invested in short-term investments in 2018, offset by the maturity of $33.0 million related to these investments, and by $1.3 million of cash acquired in 2017 as a result of the Merger.

Financing Activities

Net cash provided by financing activities was $41.9 million for the year ended December 31, 2018, compared to $52.5 million during the year ended December 31, 2017. The change in cash provided by financing activities was primarily driven by lower net proceeds received from financing activities during 2018. In 2018, we received net proceeds from (a) the sale of our common stock in a public offering of $37.9 million, (b) the sale of our common stock pursuant to the ATM Agreement of $2.7 million, and (c) the issuance of our common stock to LLS of $0.9 million. In 2017, we received net proceeds from (a) the sale of our common stock in our 2017 private financing of $39.5 million, (b) the sale of our common stock pursuant to the ATM Agreement of $7.5 million, and (c) the issuance of notes payable of $10.0 million, partially offset by payments of principal on notes payable of $4.7 million.

Contractual Obligations and Commitments

As of December 31, 2018, we had no material commitments other than the liabilities reflected and commitments disclosed in our consolidated financial statements.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements and do not have any holdings in variable interest entities.

Implications of Being an Emerging Growth Company

In April 2012, the Jumpstart Our Business Startups Act, or the JOBS Act, was enacted. Section 107 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.

We are an “emerging growth company,” as defined in the JOBS Act. We will remain an emerging growth company until the earlier of (1) the end of the first fiscal year following the fifth anniversary of our initial public offering, or December 31, 2019, (2) the beginning of the first fiscal year after our annual gross revenue is $1.07 billion or more, (3) the date on which we have, during the previous three-year period, issued more than $1.0 billion in non-convertible debt securities and (4) as of the end of

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any fiscal year in which the market value of our common stock held by non-affiliates exceeded $700 million as of the end of the second quarter of that fiscal year.

For as long as we remain an “emerging growth company,” we may take advantage of certain exemptions from various reporting requirements that are applicable to public companies that are not “emerging growth companies” including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation and financial statements in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote to approve executive compensation and shareholder approval of any golden parachute payments not previously approved.

An emerging growth company can also choose to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have elected to avail ourselves of the extended transition period for adopting new or revised accounting standards. As a result of this election, our financial statements may not be comparable to companies that comply with public company effective dates.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements and supplemental data required by this item are set forth at the pages indicated in Part IV, Item 15(a)(1) of this Annual Report.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

Not applicable.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file under the Exchange Act, is recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosures. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Under the supervision and with the participation of our principal executive officer, principal financial officer, and other senior management personnel, we evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this Annual Report on Form 10-K. Based on this evaluation, our principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were effective as of the end of the period covered by this Annual Report on Form 10-K.

Management’s Report on Internal Control Over Financial Reporting

Internal control over financial reporting refers to the process designed by, or under the supervision of, our principal officer and principal financial officer, and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that: (1) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets, (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors, and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the company’s assets that could have a material effect on the financial statements.

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Internal control over financial reporting cannot provide absolute assurance of achieving financial reporting objectives because of its inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk.

Management is responsible for establishing and maintaining adequate internal control over our financial reporting, as such term is defined in Rule 13a-15(f) under the Exchange Act. Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of the end of the period covered by this Annual Report on Form 10-K. Management used the framework set forth in the report entitled “Internal Control — Integrated Framework (2013 Framework)” published by the Committee of Sponsoring Organizations of the Treadway Commission to evaluate the effectiveness of our internal control over financial reporting. Based on its evaluation, management concluded that our internal control over financial reporting was effective at a reasonable level of assurance as of December 31, 2018, the end of our most recent fiscal year.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting that occurred during our most recent fiscal quarter that have materially affected, or are reasonably likely to materially effect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Not applicable.

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PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE

The following table lists the names and ages as of March 14, 2019 and positions of the individuals who are currently serving as our executive officers and directors:

Name	Age	Position(s)
Executive Officers		
William S. Marshall, Ph.D.	55	President, Chief Executive Officer, and Director
Jason A. Leverone	45	Chief Financial Officer, Secretary, and Treasurer
Adam S. Levy	40	Chief Business Officer
Paul D. Rubin, M.D.	65	Executive Vice President, Research and Development
Non-Employee Directors		
Christopher J. Bowden, M.D.	57	Director
Jeffrey S. Hatfield	61	Director
Thomas E. Hughes, Ph.D.	59	Director
Kevin Koch, Ph.D.	58	Director
Arlene M. Morris	67	Director
Joseph L. Turner	67	Director

Executive Officers

William S. Marshall, Ph.D. Dr. Marshall has served as our president and chief executive officer and as a director since February 2017. Prior to joining us, Dr. Marshall was the president, chief executive, and a director of Private Miragen since the company was founded in September 2007. Prior to founding Private Miragen, Dr. Marshall was vice president of technology and business development for bioscience at Thermo Fisher Scientific Inc., a serving science company, from April 2005 to July 2007. Dr. Marshall was one of the scientific founders of Dharmacon, Inc., a biotechnology company, which was acquired by Fisher Scientific International Inc. in April 2004, and he served as the executive vice president for research and operations and general manager of Dharmacon from August 2002 to April 2005. Prior to joining Dharmacon, Dr. Marshall served in multiple positions at Amgen, Inc., a biotechnology company, most recently as associate director of research, site head for research and head of the nucleic acid and peptide technology department. Dr. Marshall earned a B.S. in Biochemistry from the University of Wisconsin-Madison and his Ph.D. in Chemistry at the University of Colorado at Boulder.

We believe that Dr. Marshall’s role as our chief executive, prior board of director service, and extensive experience and innovations in the field of biotechnology enable him to bring a unique perspective to our board of directors. In addition, Dr. Marshall’s academic expertise and accomplishments provide the board of directors with in-depth product and field knowledge.

Jason A. Leverone. Mr. Leverone has served as our chief financial officer, secretary, and treasurer since February 2017. Prior to joining us, Mr. Leverone joined Private Miragen in November 2008 as its senior director of finance and operations and was appointed vice president, finance in March 2010. Mr. Leverone was appointed as Private Miragen’s chief financial officer in February 2012. Prior to joining Private Miragen, Mr. Leverone was senior director of finance and controller for Replidyne, Inc., a publicly-traded biotechnology company, from November 2005 to November 2008. Prior to joining Replidyne, Mr. Leverone was the corporate controller for CreekPath System, Inc., an international software development company, from September 2002 to October 2005. He commenced his professional career with the accounting firm of Ernst and Young LLP, where he last served as a senior accountant, and then Arthur Andersen LLP, where he last served as an audit manager. Mr. Leverone is a Certified Public Accountant and earned a

B.S. in Business Administration from Bryant University.

Adam S. Levy. Mr. Levy has served as our chief business officer since February 2017. Prior to joining us, Mr. Levy served as Private Miragen's chief business officer since May 2016. Prior to joining Private Miragen, Mr. Levy served as a senior vice president of healthcare investment banking at Wedbush Securities Inc. from September 2013 to May 2016. From May 2011 to August 2012, Mr. Levy was employed by Merrill Lynch, Pierce, Fenner & Smith, Incorporated as vice president of healthcare investment banking. Prior to joining Merrill Lynch, Mr. Levy served as vice president of healthcare investment banking at Wedbush from October 2009 through April 2011. Between 2000 through 2009, Mr. Levy held multiple investment banking

positions at Merrill Lynch, Pierce, Fenner & Smith, and Jefferies Group. Mr. Levy earned a B.S. in Applied Economics from Cornell University.

Paul D. Rubin, M.D. Dr. Rubin has served as our executive vice president, research and development since February 2017. Prior to joining us, Dr. Rubin served as Private Miragen's executive vice president, research and development since November 2016. Prior to joining Private Miragen, Dr. Rubin served as senior vice president, research and development and chief medical officer of Xoma Corporation, a publicly-traded biotechnology company, from November 2011 to November 2016, having joined Xoma in June 2011 as its vice president, clinical development, and chief medical officer. Prior to joining Xoma, Dr. Rubin was the chief medical officer at Funxional Therapeutics Ltd., a pharmaceutical company from February 2011 to June 2011. He served as chief executive officer of Resolvyx Pharmaceuticals, Inc. from 2007 to 2009 and president and chief executive officer of Critical Therapeutics, Inc. from 2002 to 2007. From 1996 to 2002, Dr. Rubin served as senior vice president, development, and later as executive vice president, research and development at Sepracor, Inc. From 1993 to 1996, Dr. Rubin held senior level positions at Glaxo-Wellcome Pharmaceuticals, most recently as vice president of worldwide clinical pharmacology and early clinical development. During his tenure with Abbott Laboratories from 1987 to 1993, Dr. Rubin served as vice president, immunology and endocrinology. Dr. Rubin received a B.A. from Occidental College and his M.D. from Rush Medical College. He completed his training in internal medicine at the University of Wisconsin.

Non-Employee Directors

Christopher Bowden, M.D. Dr. Bowden has served as a member of our board of directors since August 2017. Currently he is the chief medical officer at Agios Pharmaceuticals, Inc., and he has been in this role since 2014. Prior to joining Agios Pharmaceuticals, Inc., he served as vice president, product development oncology, franchise lead (Signaling Group) at Genentech, Inc., a member of the Roche Group. Dr. Bowden received his M.D. from Hahnemann University School of Medicine in Philadelphia followed by internal medicine training at Roger Williams Medical Center and Providence VA Medical Center, Rhode Island. He completed his medical oncology fellowship at the National Cancer Institute Medicine Branch and is board certified in internal medicine and medical oncology.

We believe Dr. Bowden is qualified to serve on our board of directors due to his years of experience in the biotechnology industry and substantial experience in clinical drug development, which enable him to contribute important strategic insight to our board of directors.

Jeffrey S. Hatfield. Mr. Hatfield has served as a member of our board of directors since August 2017. Mr. Hatfield is a veteran biotechnology and pharmaceutical industry leader, with over three decades of experience. Mr. Hatfield is currently the chief executive officer of Zafgen, Inc. Previously, he served as president and chief executive officer of Vitae Pharmaceuticals, Inc., until its acquisition by Allergan in 2016. Prior to working at Vitae Pharmaceuticals, Inc., Mr. Hatfield served in numerous executive capacities at Bristol-Myers Squibb Company, or BMS, including senior vice president of BMS's Immunology and Virology divisions, president of BMS Canada, and head of U.S. market access. Mr. Hatfield currently serves as a director on the boards of publicly traded biotechnology companies aTyr Pharma, Inc. and Zafgen, Inc., and has previously served as a director of Ambit Biosciences Corporation, prior to its acquisition by Daiichi Sankyo Company, Ltd., and InVivo Therapeutics, Inc. He is an adjunct professor and a dean's advisory board member for Purdue University's College of Pharmacy. He earned a B.S. degree in pharmacy from Purdue University's College of Pharmacy and an M.B.A. degree from The Wharton School at the University of Pennsylvania.

We believe Mr. Hatfield is qualified to serve on our board of directors due to his relevant industry experience in the biotechnology industry and experience in serving on public, biopharmaceutical company boards of directors, which enable him to contribute important strategic insight to our board of directors.

Thomas E. Hughes, Ph.D. Dr. Hughes has served as a member of our board of directors since February 2017 and, prior to joining our board of directors, he served as a member of Private Miragen's board of directors since September 2009. Dr Hughes serves as chief executive officer and as a director of Navitor Pharmaceuticals, Inc., a privately-held biopharmaceutical company, having joined the company in September 2018. Prior to that, Dr. Hughes served in varying capacities at Zafgen, Inc., a publicly-traded biopharmaceutical company, as the chief executive officer and as a director from October 2008 through October 2017, as president from October 2008 until June 2014, and as chief scientific officer and president from October 2017 through August 2018. From 1987 to 2008, Dr. Hughes held several positions at Novartis AG (formerly Sandoz Pharmaceuticals), including vice president and global head of the cardiovascular and metabolic diseases therapeutic area at the Novartis Institutes for BioMedical Research in Cambridge, MA. Dr. Hughes also serves as a member of the strategic advisory board for Broadview Ventures, an early-stage investment company. Dr. Hughes earned a Ph.D. in nutritional biochemistry from Tufts University, an M.S. in Zoology from Virginia Polytechnic Institute and State University and a B.A. in biology from Franklin and Marshall College.

We believe Dr. Hughes is qualified to serve on our board of directors due to his years of experience in the biotechnology industry and service on both public and private boards of directors of biopharmaceutical companies, which enable him to contribute important strategic insight to our board of directors.

Kevin Koch, Ph.D. Dr. Koch has served as a member of our board of directors since February 2017 and, prior to joining our board of directors, he served as a member of Private Miragen's board of directors since July 2016. Dr. Koch has served as the president and chief executive officer of Edgewise Therapeutics since July 2017 and has served as a venture partner at OrbiMed Advisors, LLC since May 2016. Prior to joining OrbiMed, Dr. Koch acted as a consultant in the biotech industry from September 2015 to May 2016. Prior to acting as a consultant, Dr. Koch served as the senior vice president, drug discovery, chemical and molecular therapeutics, at Biogen, Inc. from December 2013 to September 2015. Prior to joining Biogen, Dr. Koch founded Array BioPharma Inc., a publicly-traded biopharmaceutical company, and served as its president, chief scientific officer, and a member of its board of directors from May 1998 to November 2013. Prior to forming Array, Dr. Koch was an associate director of medicinal chemistry and project leader for the protease inhibitor and new technologies group for Amgen Inc. from 1995 to 1998. From 1988 until 1995, Dr. Koch held various research positions within the Central Research Division of Pfizer, Inc., including senior research investigator and senior research scientist. Dr. Koch earned a B.S. in chemistry and in biochemistry from the State University of New York at Stony Brook and a Ph.D. in synthetic organic chemistry from the University of Rochester.

We believe Dr. Koch is qualified to serve on our board of directors due to his years of experience in the biotechnology industry and service on both public and private boards of biopharmaceutical companies, which enable him to contribute important strategic insight to our board of directors.

Arlene M. Morris. Ms. Morris has served as a member of our board of directors since January 2018. Ms. Morris has served as chief executive officer at Willow Advisors, LLC, a consultancy advising biotech companies on financing, strategy, and business development, since May 2015. From April 2012 until May 2015, Ms. Morris served as the chief executive officer of Syndax Pharmaceuticals, Inc., a privately-held oncology company focused on the development and commercialization of therapies for treatment-resistant cancers. She also served as a member of the Syndax Pharmaceuticals board of directors from June 2011 until May 2015. From 2003 to January 2011, Ms. Morris served as the president, chief executive officer, and a member of the board of directors of Affymax, Inc., a publicly-traded biotechnology company. Ms. Morris also held various management and executive positions at Clearview Projects, Inc., a corporate advisory firm; Coulter Pharmaceutical, Inc., a publicly-traded pharmaceutical company; Scios Inc., a publicly-traded biopharmaceutical company; and Johnson & Johnson, a publicly-traded healthcare company. She is currently a member of the board of directors of Viveve Medical, Inc., a publicly-traded medical device company; Palatin Technologies, a publicly-traded biotechnology company; and Neovacs, SA, a French publicly-traded biotechnology company. She was a director of Biodel Inc., a publicly-traded specialty pharmaceutical company, from 2015 until its merger with Albireo Limited in 2016; and Dimension Therapeutics, a publicly-traded gene therapy company, until it was acquired by Ultragenyx in 2017. She currently serves as a board chair for the Foundation for Research and Development at the Medical University of South Carolina and as a trustee of Carlow University. Ms. Morris received a B.A. in biology and chemistry from Carlow College.

We believe Ms. Morris is qualified to serve on our board of directors due to her relevant industry experience and a breadth of expertise from past and continued service on the boards of directors of publicly traded biotechnology companies, which enable her to contribute important strategic insight to our board of directors.

Joseph L. Turner. Mr. Turner has served as a member of our board of directors since February 2017. Mr. Turner currently serves on the board of directors of Rainier Therapeutics, Inc. Prior to joining our board of directors, Mr. Turner served on the boards of directors and was the chair of the audit committees of Corcept Therapeutics, Inc., a publicly-traded pharmaceutical company, from 2012 to May 2016, Kythera Biopharmaceuticals, Inc., a

publicly-traded pharmaceutical company, from 2008 until Kythera's acquisition by Allergan Inc. October 2015, and Sophiris Bio, a publicly-traded pharmaceutical company from 2013 to May 2016. From July 2010 until its acquisition by Grupo Ferrer Internacional, S.A. in June 2016, Mr. Turner served on the board of directors and as a chair of the audit committee of Alexza Pharmaceuticals, Inc., a publicly-traded pharmaceutical company. In 2012, Mr. Turner served on the board of directors and as chair of the audit committee of Allos Therapeutics, Inc., a publicly-traded pharmaceutical company, until its acquisition by Spectrum Pharmaceuticals Inc. in September 2012. From 2010 through 2012, he served on the board of directors and as a member of the audit committee of QLT Inc., a publicly-traded biotechnology company. In 2008, Mr. Turner served as a director and member of the audit committee of SGX Pharmaceuticals Inc., a publicly-traded pharmaceutical company. Mr. Turner served as chief financial officer at Myogen, Inc., a publicly-traded biopharmaceutical company, from 1999 until it was acquired by Gilead Sciences in 2006. Previously, Mr. Turner was the chief financial officer at Centaur Pharmaceuticals, Inc. and served as chief financial officer and vice president, finance and administration at Cortech, Inc. Since 2009, Mr. Turner has also served on the board of managers of Swarthmore College where

at various times he has served on its executive committee, finance committee, audit committee, academic affairs committee, student affairs committee, and property committee. In 2013 until 2015, Mr. Turner served on the board of directors of the Linda Crnic Institute for Down Syndrome at the University of Colorado Medical School. Mr. Turner has an M.B.A. from the University of North Carolina at Chapel Hill, an M.A. in molecular biology from the University of Colorado and a B.A. in chemistry from Swarthmore College.

We believe Mr. Turner is qualified to serve on our board of directors due to his years of service on both public and private boards of directors of pharmaceutical companies, including service on audit committees and extensive finance experience, which enable him to contribute important strategic insight to our board of directors.

Directors Who Resigned During the Year Ended December 31, 2018

Bruce L. Booth, Ph.D. Dr. Booth served as a member of our board of directors from February 2017 through December 2018. Prior to joining our board of directors, Dr. Booth served as a member of Private Miragen's board of directors since September 2007. Dr. Booth joined Atlas Venture in 2005, serving as a partner of the company. Prior to joining Atlas Venture, from 2004 to 2005, Dr. Booth was a principal at Caxton Health Holdings L.L.C., a healthcare-focused investment firm. Prior to joining Caxton, from 1999 to 2004, Dr. Booth was an associate principal at McKinsey & Company, a global strategic management consulting firm. Dr. Booth serves on the board of Zafgen, Inc., a publicly-traded biopharmaceutical company, and several privately-held companies. Dr. Booth earned a Ph.D. in molecular immunology from Oxford University's Nuffield Department of Medicine and a B.S. in biochemistry from Pennsylvania State University.

We believe Dr. Booth was qualified to serve on our board of directors due to his years of investment in the healthcare industry and his leadership experience serving on the boards of directors of both private and public companies. Dr Booth's resignation did not result from a disagreement on any matter relating to the company's operations, policies, or practices.

Audit Committee and Financial Expert

The audit committee of our board of directors was established by our board of directors in accordance with Section 3(a)(58)(A) of the Exchange Act to oversee our corporate accounting and financial reporting processes and audits of our financial statements. Our audit committee is currently composed of Mr. Turner, who serves as chairman, and Ms. Morris and Mr. Hatfield, each of whom our board of directors has determined satisfies Nasdaq and SEC independence requirements. Our board of directors has also determined that Mr. Turner qualifies as an "audit committee financial expert," as defined in applicable SEC rules.

Section 16(a) Beneficial Ownership Reporting Compliance

Section 16(a) of the Exchange Act requires our directors and executive officers, and persons who own more than ten percent of a registered class of our equity securities, to file with the SEC initial reports of ownership within 10 days after he or she becomes a beneficial owner, director or officer and reports of changes in ownership of our common stock and other equity securities within two business days after the transaction is executed. Our officers, directors and greater than ten percent stockholders are required by SEC regulations to furnish us with copies of all Section 16(a) forms they file. To our knowledge, based solely on a review of the copies of such reports furnished to us and written representations that no other reports were required, during the fiscal year ended December 31, 2018, all Section 16(a) filing requirements applicable to our officers, directors and greater than ten percent beneficial owners were in compliance.

Code of Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers, and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on our website, which is located at www.miragen.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

ITEM 11. EXECUTIVE COMPENSATION

Our named executive officers for the year ended December 31, 2018, which consist of each person who served as our principal executive officer for the year ended December 31, 2018, and our two other most highly compensated executive officers for the year ended December 31, 2018, consisted of the following:

Officer	Title
William S. Marshall, Ph.D.	Chief Executive Officer and Director (Principal Executive Officer)
Adam S. Levy	Chief Business Officer
Paul D. Rubin, M.D.	Executive Vice President, Research and Development

Summary Compensation Table

The following table sets forth the information as to compensation paid to or earned by our named executive officers.

Name	Principal Position	Fiscal Year	Salary	Bonus	Stock Award(s)(1)	Option Award(s)(1)	All Other Compensation	Total
William S. Marshall, Ph.D.	Chief Executive Officer and President	2018	$490,000	$147,000	$—	$1,350,005	$—	$1,987,005
		2017	$400,000	$180,000	$—	$1,609,483	$—	$2,189,483
Adam S. Levy	Chief Business Officer	2018	$345,000	$82,800	$—	$440,818	$—	$868,618
		2017	$300,000	$108,000	$—	$704,149	$—	$1,112,149
Paul D. Rubin, M.D.	Executive Vice President, Research and Development	2018	$410,000	$98,400	$—	$468,369	$—	$976,769
		2017	$395,000	$142,200	$—	$704,149	$—	$1,241,349

(1) Amounts shown in this column consist of the aggregate grant date fair value of stock awards or options to purchase common stock that were granted during the applicable fiscal year, computed in accordance with FASB ASC Topic 718, "Stock Compensation". Our methodology, including our underlying estimates and assumptions used in calculating these values, is set forth in "Note 11. Share-Based Compensation" to our accompanying audited consolidated financial statements.

Current Executive Officer Employment Agreements

Marshall Employment Agreement

In December 2016, Private Miragen entered into an employment agreement with Dr. Marshall to be effective, and which we assumed, upon the closing of the Merger. Under this employment agreement, Dr. Marshall is entitled to an annual base salary (subject to periodic review and adjustment by our board of directors or compensation committee) of $490,000 and a discretionary annual cash bonus equal to 50% of Dr. Marshall's then effective base salary (subject to review and adjustment in the sole discretion of the board of directors or our compensation committee of the board of directors). Dr. Marshall is also eligible to participate in, subject to applicable eligibility requirements, all of our benefits plans and fringe benefits and programs that may be provided to our senior executives of from time to time. During 2018, Dr. Marshall was awarded the following additional compensation, which is also summarized in the compensation table above:

- cash bonuses totaling $147,000, and
- options to purchase 245,000 shares common stock at an exercise price of $7.50, exercisable over a term of four years, of which 6.25% of the shares vested on April 30, 2018, and the remaining 93.75% of the shares vest thereafter in 45 monthly installments.

The employment agreement provides that either party may terminate the agreement at-will. In addition, the agreement provides that if we terminate Dr. Marshall's employment without cause or Dr. Marshall resigns for good reason, Dr. Marshall will be eligible to receive the following severance benefits: (i) an amount equal to 12 months of his annual base salary, less applicable deductions, payable in accordance with our normal payroll schedule; (ii) the vesting of the equivalent of 12 months on all of Dr. Marshall's stock options or other equity awards that were outstanding as of the effective date of Dr. Marshall's employment

agreement; and (iii) 12 months of continued health coverage. Although, if such termination or resignation occurs within one month prior to or 12 months following a change of control, Dr. Marshall will be eligible to receive the following severance benefits: (i) an amount equal to 24 months of his annual base salary, less applicable deductions, payable in accordance with our normal payroll schedule; (ii) the vesting in full of all of his then outstanding stock options or other equity awards then outstanding and subject to time-based vesting; and (iii) 12 months of continued health coverage.

The following definitions have been adopted in Dr. Marshall's employment agreement:

- "cause" means (i) Dr. Marshall's commission of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) Dr. Marshall's attempted commission of, or participation in, a fraud or act of dishonesty against us; (iii) Dr. Marshall's intentional, material violation of any contract or agreement between Dr. Marshall and us or any statutory duty Dr. Marshall owes to us, in each case, which remains uncured for 30 days after we provide written notice of such action or conduct to Dr. Marshall; (iv) Dr. Marshall's unauthorized use or disclosure of our confidential information or trade secrets; or (v) Dr. Marshall's gross misconduct which remains uncured for 30 days after we provide written notice of such action or conduct to Dr. Marshall.

- "good reason" means the occurrence, without Dr. Marshall's consent, of any one or more of the following: (i) a material reduction in his base salary of 10% or more (unless such reduction is pursuant to a salary reduction program applicable generally to our similarly situated executives); (ii) a material reduction in Dr. Marshall's authority, duties or responsibilities; (iii) a relocation of Dr. Marshall's principal place of employment to a place that increases Dr. Marshall's one-way commute by more than 25 miles; or (iv) material breach by us of any material provision of Dr. Marshall's employment agreement.

All severance benefits payable to Dr. Marshall under his employment agreement are subject to him signing, not revoking, and complying with a release of claims.

Levy Employment Agreement

In December 2016, Private Miragen entered into an employment agreement with Mr. Levy to be effective, and which we assumed, upon the closing of the Merger. Under this employment agreement, Mr. Levy is entitled to an annual base salary (subject to periodic review and adjustment by our board of directors or compensation committee) of $345,000 and a discretionary annual cash bonus equal to 40% of Mr. Levy's then effective base salary (subject to review and adjustment in the sole discretion of the board of directors or our compensation committee of the board of directors). Mr. Levy is also eligible to participate in, subject to applicable eligibility requirements, all of our benefits plans and fringe benefits and programs that may be provided to our senior executives of from time to time. During 2018, Mr. Levy was awarded the following additional compensation, which is also summarized in the compensation table above:

- cash bonuses totaling $82,800, and
- options to purchase 80,000 shares common stock at an exercise price of $7.50, exercisable over a term of four years, of which 6.25% of the shares vested on April 30, 2018, and the remaining 93.75% of the shares vest thereafter in 45 monthly installments.

The employment agreement provides that either party may terminate the agreement at-will. In addition, the agreement provides that if we terminate Mr. Levy's employment without cause or Mr. Levy resigns for good reason, Mr. Levy will be eligible to receive the following severance benefits: (i) an amount equal to 12 months of his annual base salary, less applicable deductions, payable in accordance with our normal payroll schedule; (ii) the vesting of the equivalent of 12 months on all of Mr. Levy's stock options or other equity awards that were outstanding as of the effective date of Mr. Levy's employment agreement; and (iii) 12 months of continued health coverage. Although, if such termination or resignation occurs within one month prior to or 12 months following a change of control, Mr. Levy will be eligible to

receive the following severance benefits: (i) an amount equal to 12 months of his annual base salary, less applicable deductions, payable in accordance with our normal payroll schedule; (ii) the vesting in full of all of Mr. Levy's then outstanding stock options or other equity awards subject to time-based vesting; and (iii) twelve months of continued health coverage.

The following definitions have been adopted Mr. Levy's employment agreement:

- "cause" means (i) Mr. Levy's commission of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) Mr. Levy's attempted commission of, or participation in, a fraud or act of dishonesty against us; (iii) Mr. Levy's intentional, material violation of any contract or agreement between Mr. Levy and us or any statutory duty Mr. Levy owes to us, in each case, which remains uncured for 30 days after we provide written notice of such action or conduct to Mr. Levy; (iv) Mr. Levy's unauthorized use or disclosure of our confidential information or trade secrets; or (v) Mr. Levy's gross misconduct which remains uncured for 30 days after we provide written notice of such action or conduct to Mr. Levy.

- "good reason" means the occurrence, without Mr. Levy's consent, of any one or more of the following: (i) a material reduction in his base salary of 10% or more (unless such reduction is pursuant to a salary reduction program applicable generally to our similarly situated executives); (ii) a material reduction in Mr. Levy's authority, duties or responsibilities; (iii) a relocation of Mr. Levy's principal place of employment to a place that increases Mr. Levy's one-way commute by more than 25 miles; or (iv) material breach by us of any material provision of Mr. Levy's employment agreement.

All severance benefits payable to Mr. Levy under his employment agreement are subject to him signing, not revoking, and complying with a release of claims.

Rubin Employment Agreement

In December 2016, Private Miragen entered into an employment agreement with Dr. Rubin to be effective, and which we assumed, upon the closing of the Merger. Under this employment agreement, Dr. Rubin is entitled to an annual base salary (subject to periodic review and adjustment by our board of directors or compensation committee) of $410,000 and a discretionary annual cash bonus equal to 40% of Dr. Rubin's then effective base salary (subject to review and adjustment in the sole discretion of the board of directors or our compensation committee of the board of directors). Dr. Rubin is also eligible to participate in, subject to applicable eligibility requirements, all of our benefits plans and fringe benefits and programs that may be provided to our senior executives of from time to time. During 2018, Dr. Rubin was awarded the following additional compensation, which is also summarized in the compensation table above:

- cash bonuses totaling $98,400, and
- options to purchase 85,000 shares common stock at an exercise price of $7.50, exercisable over a term of four years, of which 6.25% of the shares vested on April 30, 2018, and the remaining 93.75% of the shares vest thereafter in 45 monthly installments.

The employment agreement provides that either party may terminate the agreement at-will. In addition, the agreement provides that if we terminate Dr. Rubin's employment without cause or Dr. Rubin resigns for good reason, Dr. Rubin will be eligible to receive the following severance benefits: (i) an amount equal to 12 months of his annual base salary, less applicable deductions, payable in accordance with our normal payroll schedule; (ii) the vesting of the equivalent of 12 months on all of Dr. Rubin's stock options or other equity awards that were outstanding as of the effective date of Dr. Rubin's employment agreement; and (iii) 12 months of continued health coverage. Although, if such termination or resignation occurs within one month prior to or 12 months following a change of control, Dr. Rubin will be eligible to receive the following severance benefits: (i) an amount equal to 12 months of his annual base salary, less applicable deductions, payable in accordance with our normal payroll schedule; (ii) the vesting in full of all of Dr. Rubin's then outstanding stock options or other equity awards subject to time-based vesting; and (iii) twelve months of continued health coverage.

The following definitions have been adopted in Dr. Rubin's employment agreement:

"cause" means (i) Dr. Rubin's commission of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) Dr. Rubin's attempted commission of, or participation in, a fraud or act of dishonesty against us; (iii) Dr. Rubin's intentional, material violation of any contract or agreement between Dr. Rubin and us or any statutory duty Dr. Rubin owes to us, in each case, which remains uncured for 30 days after we provide written notice of such action or conduct to Dr. Rubin; (iv) Dr. Rubin's unauthorized use or disclosure of our confidential information or trade secrets; or (v) Dr. Rubin's gross misconduct which remains uncured for 30 days after we provide written notice of such action or conduct to Dr. Rubin.

- "good reason" means the occurrence, without Dr. Rubin's consent, of any one or more of the following: (i) a material reduction in his base salary of 10% or more (unless such reduction is pursuant to a salary reduction program applicable

generally to our similarly situated executives); (ii) a material reduction in Dr. Rubin's authority, duties or responsibilities; (iii) a relocation of Dr. Rubin's principal place of employment to a place that increases Dr. Rubin's one-way commute by more than 25 miles; or (iv) material breach by us of any material provision of Dr. Rubin's employment agreement.

All severance benefits payable to Dr. Rubin under his employment agreement are subject to him signing, not revoking, and complying with a release of claims.

Outstanding Equity Awards at December 31, 2018

The following table provides information about the number of outstanding equity awards held by our named executive officers at December 31, 2018.

Name	Grant Date	Vesting Commencement Date		Number of securities underlying unexercised options exercisable	Number of securities underlying unexercised options unexercisable	Option exercise price	Option Expiration Date
William S. Marshall, Ph.D.	6/15/2012	6/15/2012	(1)	230,968	—	$1.22	6/13/2022
	2/22/2016	2/22/2016	(1)	111,060	45,731	$1.05	2/19/2026
	2/16/2017	2/16/2017	(1)	91,666	108,334	$11.01	2/16/2027
	1/31/2018	1/31/2018	(2)	56,145	188,855	$7.50	1/30/2028
Adam S. Levy	6/15/2016	5/16/2016	(3)	104,839	57,494	$1.05	6/13/2026
	2/16/2017	2/16/2017	(1)	40,104	47,396	$11.01	2/16/2027
	1/31/2018	1/31/2018	(2)	18,333	61,667	$7.50	1/30/2028
Paul D. Rubin, M.D.	11/30/2016	11/16/2016	(3)	105,685	97,232	$5.69	11/30/2026
	2/16/2017	2/16/2017	(1)	40,104	47,396	$11.01	2/16/2027
	1/31/2018	1/31/2018	(2)	19,479	65,521	$7.50	1/30/2028

(1) The option vests as to 1/48 of the shares in monthly installments measured from vesting commencement date.

(2) 6.25% of the shares vested on April 30, 2018, and the remaining 93.75% of the shares vest thereafter in 45 monthly installments.

(3) Twenty-five percent of the shares subject to the option vest on the first anniversary of the vesting commencement date, and the remainder vest thereafter in 36 monthly installments.

Employee Benefit Plans

2016 Equity Incentive Plan

Purpose

Our 2016 Equity Incentive Plan, or the 2016 Plan, was adopted by us, and approved by our stockholders in connection with the Merger. The 2016 Plan is designed to secure and retain the services of our employees, directors, and consultants, provide incentives for such, directors and consultants to exert maximum efforts for our success and to provide a means by our employees, directors and consultants may be given an opportunity to benefit from increases in the value of its common stock. The 2016 Plan was adopted to replace and supersede our 2014 Stock Incentive Plan, or the 2014 Plan.

As of December 31, 2018, there were outstanding stock options to purchase 1,887,617 shares of our common stock under the 2016 Plan.

Types of Awards

The terms of the 2016 Plan provide for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, other stock awards, and performance awards that may be settled in cash, stock, or other property.

Shares Available for Awards

The aggregate number of shares of our common stock that may be issued under the 2016 Plan, or the Share Reserve, will not exceed 4,182,404 shares, which number is the sum of: (i) 1,681,294 shares, plus (ii) the number of shares subject to outstanding stock awards that were granted under the Private Miragen 2008 Equity Incentive Plan, or the Miragen 2008 Plan, that, from and after the closing date of the Merger, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to meet a contingency or condition required to vest such shares, or are reacquired, withheld or not issued to satisfy a tax withholding obligation in connection with an award or to satisfy the purchase price or exercise price of a stock award, if any, as such shares become available from time to time, plus (iii) shares from automatic increases to the share reserve, as described in more detail below. In accordance with the 2016 Plan, the share reserve will automatically increase on January 1 of each year, for a period of not more than ten years, commencing on January 1 of the year following the year in which the effective date of the 2016 Plan occurs, and ending on (and including) January 1, 2026, in an amount equal to 4% of the shares of common stock outstanding on December 31st of the preceding calendar year; however the board of directors or compensation committee may act prior to January 1 of a given year to provide that there will be no January 1st increase in the share reserve for such year or that the increase in the share reserve for such year will be a lesser number of shares of common stock than would otherwise occur pursuant to the automatic increase. On January 1, 2018 and 2019, the share reserve had automatic increases of 902,720 and 1,233,578 shares, respectively.

The following shares of common stock will become available again for issuance under the 2016 Plan: (i) any shares subject to a stock award that are not issued because such stock award expires or otherwise terminates without all of the shares covered by such stock award having been issued; (ii) any shares subject to a stock award that are not issued because such stock award is settled in cash; (iii) any shares issued pursuant to a stock award that are forfeited back to or repurchased by us because of the failure to meet a contingency or condition required for the vesting of such shares; and (iv) any shares reacquired by us in satisfaction of tax withholding obligations on a stock award or as consideration for the exercise or purchase price of a stock award.

Eligibility

All of our employees and non-employee directors are eligible to participate in the 2016 Plan and may receive all types of awards other than incentive stock options. Incentive stock options may be granted under the 2016 Plan only to our employees (including officers) and employees of our affiliates.

Section 162(m) Limits

Under the 2016 Plan, subject to adjustment for specified changes in our capitalization, no participant will be eligible to be granted performance-based compensation during any calendar year more than: (i) a maximum of 1,500,000 shares of common stock subject to stock options and stock appreciation rights whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the fair market value of a share of common stock on the date of grant; (ii) a maximum of 1,500,000 shares of common stock subject to performance stock awards; and (iii) a maximum of $3,000,000 subject to performance cash awards. These limits are designed to allow us to grant awards that are intended to be exempt from the $1.0 million limitation on the income tax deductibility of compensation paid per covered employee imposed by Section 162(m) of the Code and will not apply to awards that our board of directors determines will not be treated as performance-based compensation.

Non-Employee Director Compensation Limit

Under the 2016 Plan, the maximum number of shares of common stock subject to stock awards granted under the 2016 Plan or otherwise during any one calendar year to any non-employee director, taken together with any cash fees paid us to such non-employee director during such calendar year for services on its board of directors, will not exceed $500,000 in total value (calculating the value of any such stock awards based on the grant date fair value of such stock awards for financial reporting purposes), or, with respect to the calendar year in which a non-employee director is first appointed or elected to our board of directors, $1,000,000.

Administration

The 2016 Plan is administered by our board of directors, which may in turn delegate authority to administer the 2016 Plan to a committee. Our board of directors has delegated concurrent authority to administer the 2016 Plan to its compensation committee, but may, at any time, revest in itself some or all of the power delegated to its compensation committee. Our board of directors and its compensation committee are each considered to be a Plan Administrator for purposes of the 2016 Plan. Subject to the terms of the 2016 Plan, the Plan Administrator may determine the recipients, the types of awards to be granted, the number of shares of common stock subject to or the cash value of awards, and the terms and conditions of awards granted under the 2016 Plan, including the period of their exercisability and vesting. The Plan Administrator also has the authority to provide for accelerated exercisability and vesting of awards. Subject to the limitations set forth below, the Plan Administrator also determines the fair market value applicable to a stock award and the exercise or strike price of stock options and stock appreciation rights granted under the 2016 Plan.

The Plan Administrator may also delegate to one or more officers the authority to designate employees who are not officers to be recipients of certain stock awards and the number of shares of common stock subject to such stock awards. Under any such delegation, the Plan Administrator will specify the total number of shares of common stock that may be subject to the stock awards granted by such officer. The officer may not grant a stock award to himself or herself.

Repricing, Cancellation, and Re-Grant of Stock Awards

Under the 2016 Plan, the Plan Administrator does not have the authority to reprice any outstanding stock option or stock appreciation right by reducing the exercise or strike price of the stock option or stock appreciation right or to cancel any outstanding stock option or stock appreciation right that has an exercise or strike price greater than the then-current fair market value of a share of common stock in exchange for cash or other stock awards without obtaining the approval of our stockholders. Such approval must be obtained within 12 months prior to such an event.

Stock Options

Stock options may be granted under the 2016 Plan pursuant to stock option agreements. The 2016 Plan permits the grant of stock options that are intended to qualify as ISOs and NSOs.

The exercise price of a stock option granted under the 2016 Plan may not be less than 100% of the fair market value of the common stock subject to the stock option on the date of grant and, in some cases (see “Limitations on Incentive Stock Options” below), may not be less than 110% of such fair market value.

The term of stock options granted under the 2016 Plan may not exceed ten years and, in some cases (see “Limitations on Incentive Stock Options” below), may not exceed five years. Except as otherwise provided in a participant’s stock option agreement or other written agreement with us or one of our affiliates, if a participant’s service relationship with us or any of our affiliates, referred to herein as continuous service, terminates (other than for cause and other than upon the participant’s death or disability), the participant may exercise any vested stock options for up to three months following the participant’s termination of continuous service. Except as otherwise provided in a participant’s stock option agreement or other written agreement with us or one of our affiliates, if a participant’s continuous service terminates due to the participant’s disability or death (or the participant dies within a specified period, if any, following termination of continuous service), the participant, or his or her beneficiary, as applicable, may exercise any vested stock options for up to 12 months following the participant’s termination due to the participant’s disability or for up to 18 months following the participant’s death. Except as explicitly provided otherwise in a participant’s stock option agreement or other written agreement with us or one of our affiliates, if a participant’s continuous service is terminated for cause (as defined in the 2016 Plan), all stock options held by the participant will terminate upon the participant’s termination of continuous service and the participant will be prohibited from exercising any stock option from and

after such termination date. Except as otherwise provided in a participant's stock option agreement or other written agreement with us or one of its affiliates, the term of a stock option may be extended if the exercise of the stock option following the participant's termination of continuous service (other than for cause and other than upon the participant's death or disability) would be prohibited by applicable securities laws or if the sale of any common stock received upon exercise of the stock option following the participant's termination of continuous service (other than for cause) would violate our insider trading policy. In no event, however, may a stock option be exercised after its original expiration date.

Acceptable forms of consideration for the purchase of common stock pursuant to the exercise of a stock option under the 2016 Plan will be determined by the Plan Administrator and may include payment: (i) by cash, check, bank draft or money order payable to us; (ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board; (iii) by

delivery to us of shares of common stock (either by actual delivery or attestation); (iv) by a net exercise arrangement (for NSOs only); or (v) in other legal consideration approved by the Plan Administrator.

Stock options granted under the 2016 Plan may vest as determined by the Plan Administrator at the rate specified in the stock option agreement. Shares covered by different stock options granted under the 2016 Plan may be subject to different vesting schedules as the Plan Administrator may determine.

The Plan Administrator may impose limitations on the transferability of stock options granted under the 2016 Plan in its discretion. Generally, a participant may not transfer a stock option granted under the 2016 Plan other than by will or the laws of descent and distribution or, subject to approval by the Plan Administrator, pursuant to a domestic relations order or an official marital settlement agreement. However, the Plan Administrator may permit transfer of a stock option in a manner that is not prohibited by applicable tax and securities laws. In addition, subject to approval by the Plan Administrator, a participant may designate a beneficiary who may exercise the stock option following the participant's death.

Limitations on Incentive Stock Options

The aggregate fair market value, determined at the time of grant, of shares of common stock with respect to ISOs that are exercisable for the first time by a participant during any calendar year under all of our stock plans may not exceed $100,000. The stock options or portions of stock options that exceed this limit or otherwise fail to qualify as ISOs are treated as NSOs. No ISO may be granted to any person who, at the time of grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any affiliate unless the following conditions are satisfied:

- the exercise price of the ISO must be at least 110% of the fair market value of the common stock subject to the ISO on the date of grant; and
- the term of the ISO must not exceed five years from the date of grant.

Subject to adjustment for specified changes in capitalization, the aggregate maximum number of shares of common stock that may be issued pursuant to the exercise of ISOs under the 2016 Plan is 20,912,020 shares.

Stock Appreciation Rights

Stock appreciation rights may be granted under the 2016 Plan pursuant to stock appreciation right agreements. Each stock appreciation right is denominated in common stock share equivalents. The strike price of each stock appreciation right will be determined by the Plan Administrator but will in no event be less than 100% of the fair market value of the common stock subject to the stock appreciation right on the date of grant. The Plan Administrator may also impose restrictions or conditions upon the vesting of stock appreciation rights that it deems appropriate. The appreciation distribution payable upon exercise of a stock appreciation right may be paid in shares of our common stock, in cash, in a combination of cash and stock, or in any other form of consideration determined by the Plan Administrator and set forth in the stock appreciation right agreement. Stock appreciation rights will be subject to the same conditions upon termination of continuous service and restrictions on transfer as stock options under the 2016 Plan.

Restricted Stock Awards

Restricted stock awards may be granted under the 2016 Plan pursuant to restricted stock award agreements. A restricted stock award may be granted in consideration for cash, check, bank draft or money order payable to us, the participant's services performed for us or any of ours affiliates, or any other form of legal consideration acceptable to

the Plan Administrator. Shares of common stock acquired under a restricted stock award may be subject to forfeiture to or repurchase by us in accordance with a vesting schedule to be determined by the Plan Administrator. Rights to acquire shares of common stock under a restricted stock award may be transferred only upon such terms and conditions as are set forth in the restricted stock award agreement. A restricted stock award agreement may provide that any dividends paid on restricted stock will be subject to the same vesting conditions as apply to the shares subject to the restricted stock award. Upon a participant's termination of continuous service for any reason, any shares subject to restricted stock awards held by the participant that have not vested as of such termination date may be forfeited to or repurchased by us.

Restricted Stock Unit Awards

Restricted stock unit awards may be granted under the 2016 Plan pursuant to restricted stock unit award agreements. Payment of any purchase price may be made in any form of legal consideration acceptable to the Plan Administrator. A restricted stock

unit award may be settled by the delivery of shares of Signal common stock, in cash, in a combination of cash and stock, or in any other form of consideration determined by the Plan Administrator and set forth in the restricted stock unit award agreement. Restricted stock unit awards may be subject to vesting in accordance with a vesting schedule to be determined by the Plan Administrator. Dividend equivalents may be credited in respect of shares of common stock covered by a restricted stock unit award, provided that any additional shares credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying restricted stock unit award. Except as otherwise provided in a participant's restricted stock unit award agreement or other written agreement with us or one of our affiliates, restricted stock units that have not vested will be forfeited upon the participant's termination of continuous service for any reason.

Performance Awards

The 2016 Plan allows us to grant performance stock and cash awards, including such awards that may qualify as performance-based compensation that is not subject to the $1 million limitation on the income tax deductibility of compensation paid per covered employee imposed by Section 162(m) of the Code.

A performance stock award is a stock award that is payable (including that may be granted, may vest, or may be exercised) contingent upon the attainment of pre-determined performance goals during a performance period. A performance stock award may require the completion of a specified period of continuous service. The length of any performance period, the performance goals to be achieved during the performance period, and the measure of whether and to what degree such performance goals have been attained will be determined by the compensation committee of our board of directors, except that the Plan Administrator also may make any such determinations to the extent that the award is not intended to qualify as performance-based compensation under Section 162(m) of the Code. In addition, to the extent permitted by applicable law and the performance stock award agreement, the Plan Administrator may determine that cash may be used in payment of performance stock awards.

A performance cash award is a cash award that is payable contingent upon the attainment of pre-determined performance goals during a performance period. A performance cash award may require the completion of a specified period of continuous service. The length of any performance period, the performance goals to be achieved during the performance period, and the measure of whether and to what degree such performance goals have been attained will be determined by the compensation committee of our board of directors, except that the Plan Administrator also may make any such determinations to the extent that the award is not intended to qualify as performance-based compensation under Section 162(m) of the Code. The Plan Administrator may specify the form of payment of performance cash awards, which may be cash or other property, or may provide for a participant to have the option for his or her performance cash award to be paid in cash or other property.

In granting a performance stock or cash award intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the compensation committee of our board of directors will set a period of time, or a performance period, over which the attainment of one or more goals, or performance goals, will be measured. Within the time period prescribed by Section 162(m) of the Code (no later than the earlier of the 90th day of a performance period and the date on which 25% of the performance period has elapsed, and in any event at a time when the achievement of the performance goals remains substantially uncertain), the compensation committee of our board of directors will establish the performance goals, based upon one or more criteria, or performance criteria, enumerated in the 2016 Plan and described below. As soon as administratively practicable following the end of the performance period, the compensation committee of our board of directors will certify in writing whether the performance goals have been satisfied.

Performance goals under the 2016 Plan will be based on any one or more of the following performance criteria: (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) earnings before interest, taxes, depreciation,

amortization and legal settlements; (v) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (vi) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (vii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (viii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation, other non-cash expenses and changes in deferred revenue; (ix) total stockholder return; (x) return on equity or average stockholder's equity; (xi) return on assets, investment, or capital employed; (xii) stock price; (xiii) margin (including gross margin); (xiv) income (before or after taxes); (xv) operating income; (xvi) operating income after taxes; (xvii) pre-tax profit; (xviii) operating cash flow; (xix) sales or revenue targets; (xx) increases in revenue or product revenue; (xxi) expenses and cost reduction goals; (xxii) improvement in or attainment of working capital levels; (xxiii) economic value added (or an equivalent metric); (xxiv) market share; (xxv) cash flow; (xxvi) cash flow per share; (xxvii) cash balance; (xxviii) cash burn; (xxix) cash collections; (xxx) share price performance; (xxxi) debt reduction; (xxxii) implementation or completion of projects or processes (including, without limitation, clinical trial initiation, clinical trial

enrollment and dates, clinical trial results, regulatory filing submissions, regulatory filing acceptances, regulatory or advisory committee interactions, regulatory approvals, new and supplemental indications for existing products, and product supply); (xxxiii) stockholders' equity; (xxxiv) capital expenditures; (xxxv) debt levels; (xxxvi) operating profit or net operating profit; (xxxvii) workforce diversity; (xxxviii) growth of net income or operating income; (xxxix) billings; (xl) bookings; (xli) employee retention; (xlii) initiation of phases of clinical trials and/or studies by specific dates; (xliii) acquisition of new customers, including institutional accounts; (xliv) customer retention and/or repeat order rate; (xlv) number of institutional customer accounts (xlvi) budget management; (xlvii) improvements in sample and test processing times; (xlviii) regulatory milestones; (xlix) progress of internal research or clinical programs; (l) progress of partnered programs; (li) partner satisfaction; (lii) milestones related to samples received and/or tests run; (liii) expansion of sales in additional geographies or markets; (liv) research progress, including the development of programs; (lv) submission to, or approval by, a regulatory body (including, but not limited to the FDA) of an applicable filing or a product; (lvi) timely completion of clinical trials; (lvii) milestones related to samples received and/or tests or panels run; (lviii) expansion of sales in additional geographies or markets; (lix) research progress, including the development of programs; (lx) patient samples processed and billed; (lxi) sample processing operating metrics (including, without limitation, failure rate maximums and reduction of repeat rates); (lxii) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property); (lxiii) preclinical development related to compound goals; (lxiv) customer satisfaction; and (lxv) and to the extent that an award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by our board of directors.

Performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. The compensation committee our board of directors (or, to the extent that an award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Plan Administrator) is authorized to make appropriate adjustments in the method of calculating the attainment of performance goals for a performance period as follows; provided, however, that to the extent that an award is intended to qualify as "performance-based compensation" under Section 162(m) of the Code, any such adjustment may be made only if such adjustment is objectively determinable and specified in the award agreement at the time the award is granted or in such other document setting forth the performance goals for the award at the time the performance goals are established: (i) to exclude restructuring and/or other nonrecurring charges; (ii) to exclude exchange rate effects; (iii) to exclude the effects of changes to U.S. GAAP; (iv) to exclude the effects of any statutory adjustments to corporate tax rates; (v) to exclude the effects of items that are unusual in nature or occur infrequently as determined under U.S. GAAP; (vi) to exclude the dilutive effects of acquisitions or joint ventures; (vii) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (viii) to exclude the effect of any change in the outstanding shares of our common stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (ix) to exclude the effects of stock based compensation and the award of bonuses under our bonus plans; (x) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under U.S. GAAP; and (xi) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under U.S. GAAP.

In addition, the compensation committee of our board of directors (or, to the extent that an award is not intended to qualify as "performance-based compensation" under Section 162(m) of the Code, the Plan Administrator) retains the discretion to reduce or eliminate the compensation or economic benefit due upon the attainment of any performance goals and to define the manner of calculating the performance criteria it selects to use for a performance period.

Other Stock Awards

Other forms of stock awards valued in whole or in part by reference to, or otherwise based on, common stock may be granted either alone or in addition to other stock awards under the 2016 Plan. The Plan Administrator will have sole and complete authority to determine the persons to whom and the time or times at which such other stock awards will be granted, the number of shares of common stock to be granted and all other terms and conditions of such other stock awards.

Clawback Policy

Awards granted under the 2016 Plan will be subject to recoupment in accordance with any clawback policy that the we are required to adopt pursuant to the listing standards of any national securities exchange or association on which our securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law. In addition, the Plan Administrator may impose other clawback, recovery or recoupment provisions in an award agreement as the Plan Administrator determines necessary or appropriate, including a reacquisition right in respect of previously acquired shares of common stock or other cash or property upon the occurrence of cause.

Changes to Capital Structure

In the event of certain capitalization adjustments, the Plan Administrator will appropriately adjust: (i) the class(es) and maximum number of securities subject to the 2016 Plan and by which the share reserve may increase automatically each year; (ii) the class(es) and maximum number of securities that may be issued pursuant to the exercise of ISOs; (iii) the class(es) and maximum number of securities that may be awarded to any participant pursuant to Section 162(m) limits; (iv) the class and maximum number of shares that may be awarded to any non-employee director; and (v) the class(es) and number of securities and price per share of stock subject to outstanding stock awards.

Corporate Transaction

In the event of a corporate transaction (as defined in the 2016 Plan and described below), the Plan Administrator may take one or more of the following actions with respect to stock awards, contingent upon the closing or consummation of the corporate transaction, unless otherwise provided in the instrument evidencing the stock award, in any other written agreement between us or one of our affiliates and the participant or in our director compensation policy, or unless otherwise provided by the Plan Administrator at the time of grant of the stock award:

- arrange for the surviving or acquiring corporation (or its parent company) to assume or continue the stock award or to substitute a similar stock award for the stock award (including an award to acquire the same consideration paid to our stockholders pursuant to the corporate transaction);
- arrange for the assignment of any reacquisition or repurchase rights held by us in respect of common stock issued pursuant to the stock award to the surviving or acquiring corporation (or its parent company);
- accelerate the vesting (and, if applicable, the exercisability) of the stock award to a date prior to the effective time of the corporate transaction as determined by the Plan Administrator (or, if the Plan Administrator does not determine such a date, to the date that is five days prior to the effective date of the corporate transaction), with the stock award terminating if not exercised (if applicable) at or prior to the effective time of the corporate transaction; provided, however, that the Plan Administrator may require participants to complete and deliver to us a notice of exercise before the effective date of a corporate transaction, which is contingent upon the effectiveness of the corporate transaction;
- arrange for the lapse of any reacquisition or repurchase rights held by us with respect to the stock award;
- cancel or arrange for the cancellation of the stock award, to the extent not vested or not exercised prior to the effective time of the corporate transaction, and pay such cash consideration (including no consideration) as the Plan Administrator may consider appropriate; and
- cancel or arrange for the cancellation of the stock award, to the extent not vested or not exercised prior to the effective time of the corporate transaction, in exchange for a payment, in such form as may be determined by our board of directors equal to the excess, if any, of (i) the per share amount payable to holders of common stock in connection with the corporate transaction, over (ii) the per share exercise price under the applicable award. For clarity, this payment may be zero if the value of the property is equal to or less than the exercise price. In addition, any escrow, holdback, earnout or similar provisions in the definitive agreement for the corporate transaction may apply to such payment to the same extent and in the same manner as such provisions apply to the holders of common stock.

The Plan Administrator is not required to take the same action with respect to all stock awards or portions of stock awards or with respect to all participants. The Plan Administrator may take different actions with respect to the vested and unvested portions of a stock award.

In the event of a corporate transaction, unless otherwise provided in the instrument evidencing a performance cash award or any other written agreement between us or one of our affiliates and the participant, or unless otherwise provided by the Plan Administrator, all performance cash awards will terminate prior to the effective time of the corporate transaction.

For purposes of the 2016 Plan, a corporate transaction generally will be deemed to occur in the event of the consummation of: (i) a sale or other disposition of all or substantially all of our consolidated assets; (ii) a sale or other disposition of more than 50% of our outstanding securities; (iii) a merger, consolidation or similar transaction following which we are not the surviving corporation; or (iv) a merger, consolidation or similar transaction following which we are the surviving corporation but the

shares of common stock outstanding immediately prior to the transaction are converted or exchanged into other property by virtue of the transaction.

Change in Control

Under the 2016 Plan, a stock award may be subject to additional acceleration of vesting and exercisability upon or after a change in control (as defined in the 2016 Plan and described below) as may be provided in the participant's stock award agreement, in any other written agreement with us or one of our affiliates or in any director compensation policy, but in the absence of such provision, no such acceleration will occur.

2008 Equity Incentive Plan

The Miragen 2008 Plan was adopted by Private Miragen's board of directors and approved by its stockholders in May 2008, and was subsequently amended by its board of directors and stockholders, most recently in October 2015. No further awards will be made under the Miragen 2008 Plan, but all awards outstanding under the 2008 Miragen Plan as of the effective time of the Merger remain subject to the terms and conditions of the 2008 Miragen Plan.

As of December 31, 2018, there were outstanding stock options to purchase 1,639,796 shares of our common stock under the Miragen 2008 Plan.

All awards granted under the Miragen 2008 Plan that, from and after the effective date of the Merger, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to meet a contingency or condition required to vest, or are reacquired, withheld or not issued to satisfy a tax withholding obligation in connection with an award or to satisfy the exercise price of a stock award, will become available for grant under the 2016 Plan in accordance with its terms.

Stock Awards

The Miragen 2008 Plan provides for the grant of ISO, NSOs, stock appreciation rights, restricted stock awards and restricted stock unit awards (collectively, stock awards), all of which may be granted to employees, including officers, non-employee directors and consultants of Private Miragen. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants. Private Miragen only granted stock options under the Miragen 2008 Plan.

Administration

Our board of directors or the compensation committee of our board of directors may act as the administrator of the Miragen 2008 Plan. The administrator has the complete discretion to make all decisions relating to the plan and outstanding awards. The administrator has the authority to modify outstanding awards under the Miragen 2008 Plan. Subject to the terms of the Miragen 2008 Plan, the administrator has the authority to reduce the exercise or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash, or other consideration, or take any other action that is treated as a repricing under U.S. GAAP, with the consent of any adversely affected participant.

Terms of Awards

Subject to the terms of the Miragen 2008 Plan, the administrator determines the terms of all awards. The exercise price for stock options granted under the Miragen 2008 Plan may not be less than 100% of the fair market value of Miragen common stock on the grant date; however, the exercise price for an incentive stock option granted to a holder of more than 10% of Miragen's stock may not be less than 110% of such fair market value on the grant date. Options

are generally transferable only by will or the laws of descent and distribution and may be exercised during the holder's lifetime only by the holder.

The term of options granted under the Miragen 2008 Plan may not exceed ten years and will generally expire sooner if the optionee's service terminates. Options vest at the times determined by the administrator. Shares may be awarded under the terms of the Miragen 2008 Plan in consideration for services rendered to Private Miragen or sold under the terms of the Miragen 2008 Plan. Shares awarded or sold under the Miragen 2008 Plan may be fully vested at grant or subject to special forfeiture conditions or rights of repurchase as determined by the administrator.

Changes in Capitalization

If any change is made in the shares of common stock by reason of any merger, consolidation, reorganization, recapitalization, stock dividend, split up, combination of shares, exchange of shares, change in corporate structure, or otherwise, appropriate adjustments will be made by the administrator to the class and maximum number of shares reserved for issuance under the Miragen 2008 Plan, the class and maximum number of shares that may be issued upon the exercise of ISOs and the class and number of shares and price per share of stock subject to each outstanding award under the Miragen 2008 Plan. Any increase in the shares, or the right to acquire shares, as the result of such an adjustment will be subject to the same terms and conditions that apply to the award for which such increase was received.

Corporate Transaction

In the event of certain specified significant corporate transactions, outstanding stock awards shall be assumed, continued, or substituted for similar stock awards by the surviving or acquiring corporation. If any surviving or acquiring corporation fails to assume, continue, or substitute such stock awards, stock awards held by participants whose continuous service has not terminated will accelerate vesting in full prior to the corporate transaction, and all stock awards will terminate at or prior to the corporate transaction. In addition, in the event a stock award will terminate if not exercised before a corporate transaction, our board of directors may, in its sole discretion, provide that the holder of the stock award may not exercise the stock award but will receive a payment equal to the excess, if any, of (i) the value of our common stock the holder would have received upon exercise of the stock awards, over (ii) any exercise price payable by the holder in connection with the exercise.

Under the Miragen 2008 Plan, a corporate transaction is generally the consummation of (i) a sale or other disposition of all or substantially all of our consolidated assets, (ii) a sale or other disposition of at least 90% of our outstanding securities, (iii) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (iv) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

Change in Control

The Miragen 2008 Plan provides that if a change in control of us occurs and as of, or within thirteen (13) months after, the effective time of such change in control, the service of an award holder is terminated due to an involuntary termination without cause (not including death or disability), or due to a voluntary termination with good reason, then the vesting and exercisability of the holder’s awards will be accelerated in full. In addition, the administrator may provide, in an individual award agreement or in any other written agreement between a participant and us, that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change in control.

Under the Miragen 2008 Plan, a change of control is generally (i) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (ii) a consummated merger, consolidation or similar transaction involving us immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity or of its parent entity; (iii) a consummated sale, lease or exclusive license or other disposition of all or substantially of our consolidated assets; or (v) when a majority of our board of directors becomes comprised of individuals who were not serving on the board on the date of adoption of the Miragen 2008 Plan, or whose nomination, appointment, or election was not approved by a majority of the incumbent board then still in office. The Merger did not constitute a change in control for purposes of the Miragen 2008 Plan, but the change in control provisions could be triggered by a subsequent transaction.

Amendment and Termination

Our board of directors may at any time amend the Miragen 2008 Plan. However, our board of directors must obtain approval of our stockholders or any amendment requiring such approval under federal tax or federal securities laws. In addition, our board of directors may not alter or impair any award previously granted under the Miragen 2008 Plan without the consent of the holder of such award. The Miragen 2008 Plan will terminate on the earliest of ten years after the date the Miragen 2008 Plan was adopted by Private Miragen's board of directors, ten years after the date Private Miragen's stockholder approved the Miragen 2008 Plan or a date determined by our board of directors.

2018 Director Compensation

Annual Compensation

Effective upon the closing of the Merger, we assumed a non-employee director cash and equity compensation policy. During the twelve-month period following the date of each annual meeting, each non-employee director will be paid an annual cash retainer of $35,000 for his or her service on our board of directors; provided that the non-employee chairperson of the board of directors will be paid an additional annual cash retainer of $30,000.

In addition to the annual retainer described above, each non-employee director who serves as a chair or member of our audit committee, compensation committee, and nominating and corporate governance committee is paid an annual fee during the twelve-month period following the date of each annual meeting of the Company's stockholders as follows:

	Member Annual Fee (1)	Chairperson Annual Fee
Audit committee	$ 7,500	$ 15,000
Compensation committee	5,000	10,000
Nominating and corporate governance committee	3,750	7,500

(1) Annual fee paid to each non-employee director (other than the chairperson) who serves as a member of the corresponding committee of the board of directors.

Each non-employee director and committee member has the right to elect to receive all or a portion of annual compensation described above in the form of either cash, quarterly restricted common stock based on the closing price of our common stock on The Nasdaq Capital Market on the date of grant, or stock options to purchase common stock based on the Black-Scholes option-pricing model as of the date of grant. Any such election will be made before the start of the fiscal year or within thirty days of first becoming eligible to receive compensation under this policy and with any such stock options or restricted common stock elected by the directors to vest on a quarterly basis in arrears, with stock options to expire ten years from the date of grant.

Equity Awards granted upon annual re-election to the Board of Directors

In addition to the compensation described above, each member of our board of directors will receive an automatic option grant to purchase 12,000 shares of our common stock (subject to adjustment for stock splits and similar matters) at each annual meeting once re-elected with an exercise price equal to the fair market value of a share of our common stock on such date. Each equity grant will vest in full on the earlier of the one-year anniversary of the date of grant or our next annual meeting.

Equity Awards granted upon appointment to the Board of Directors

Each new director elected or appointed to our board of directors will receive an initial equity grant of options to purchase 24,000 shares of our common stock (subject to adjustment for stock splits and similar matters) upon appointment or election with an exercise price equal to the fair market value of a share of our common stock on such date. Each option grant will vest in 36 equal monthly installments.

2018 Director Compensation

The table below sets forth the compensation of our non-employee directors for the fiscal year ended December 31, 2018.

Name (1)	Fees Earned or Paid in Cash	Stock Awards (2)	Option Awards (2) (3)	All Other Compensation	Total
Bruce L. Booth, Ph.D.	$—	$—	$54,381 (4)	$—	$54,381
Christopher J. Bowden, M.D.	$40,000	$—	$57,277 (5)	$—	$97,277
Jeffrey S. Hatfield	$44,801	$—	$57,277 (5)	$—	$102,078
Thomas E. Hughes, Ph.D.	$24,375	$—	$81,653 (5)(6)	$—	$106,028
Kevin Koch, Ph.D.	$47,500	$—	$57,277 (5)	$—	$104,777
Arlene M. Morris	$38,311	$—	$228,034 (5)(7)	$—	$266,345
Joseph L. Turner	$50,000	$—	$57,277 (5)	$—	$107,277

(1) Dr. Marshall, our current president and chief executive officer, also served as a member of our board of directors in the fiscal year ended December 31, 2018. Dr. Marshall's compensation for serving as our president and chief executive officer in 2018 is reported in the Summary Compensation Table and other compensation tables set forth under "Executive Compensation." Dr. Marshall did not receive any additional compensation for his service on our board of directors.

(2) The values set forth in this column are based on the aggregate grant date fair value of the awards computed in accordance with FASB ASC Topic 718.

(3) Including these stock options, as of December 31, 2018, each person serving in 2018 as a non-employee director held the following number of stock options: Dr. Booth, 26,370; Dr. Bowden, 36,000; Mr. Hatfield, 36,000; Dr. Hughes, 53,556; Dr. Koch, 53,248; Ms. Morris, 36,000; and Mr. Turner, 48,000.

(4) On January 3, 2018, this non-employee director was granted stock options to purchases 10,191 shares of common stock at an exercise price of $9.72, which was equal to the closing price of our common stock on The Nasdaq Capital Market on the date of grant. The stock options vested in quarterly installments, fully vesting on December 31, 2018. Dr. Booth served as a member of our board of directors through December 4, 2018, as of which date 7,643 option awards granted in 2018 had previously vested and 2,548 option awards were forfeited.

(5) On June 27, 2018, each non-employee director was granted stock options to purchases 12,000 shares of common stock at an exercise price of $6.46, which was equal to the closing price of our common stock on The Nasdaq Capital Market on the date of grant. The stock options will vest in full on June 19, 2019.

(6) On January 3, 2018, this non-employee director was granted stock options to purchases 3,426 shares of common stock at an exercise price of $9.72, which was equal to the closing price of our common stock on The Nasdaq Capital Market on the date of grant. The stock options vested in quarterly installments and were fully vested on December 31, 2018.

(7) On January 3, 2018, this non-employee director was granted stock options to purchases 24,000 shares of common stock at an exercise price of $9.72, which was equal to the closing price of our common stock on The Nasdaq Capital Market on the date of grant. The stock options vest in 36 monthly installments beginning on January 3, 2018.

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

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth certain information with respect to the beneficial ownership of our capital stock as of March 1, 2019 (except where otherwise indicated) for:

- each person, or group of affiliated persons, who are known by us to beneficially own more than 5% of the outstanding shares of our capital stock;
- each of our directors as of March 1, 2019;
- each of our named executive officers as of March 1, 2019; and
- all of our current directors and executive officers of as a group.

Applicable percentages are based on 30,921,219 shares outstanding on March 1, 2019, adjusted as required by rules promulgated by the SEC. Beneficial ownership is determined under SEC rules and includes sole or shared power to vote or dispose of shares of our common stock. The number and percentage of shares beneficially owned by a person or entity also include shares of common stock subject to stock options that are currently exercisable or become exercisable within 60 days of March 1, 2019. However, these shares are not deemed to be outstanding for the purpose of computing the percentage of shares beneficially owned of any other person or entity. Except as indicated in footnotes to the table below or, where applicable, to the extent authority is shared by spouses under community property laws, the beneficial owners named in the table have, to our knowledge, sole voting and dispositive power with respect to all shares of common stock shown to be beneficially owned by them based on information provided to us by such stockholders. Unless otherwise indicated, the address for each stockholder listed is: c/o Miragen Therapeutics, Inc., 6200 Lookout Road Boulder, Colorado 80301.

Name	Number of Shares Beneficially Owned		Percentage Ownership
5% or Greater Stockholders			
Atlas Venture Fund VII, L.P.	3,142,580	(1)	10.2%
FMR, LLC	2,888,656	(2)	9.3%
Remeditex Ventures LLC	2,706,563	(3)	8.8%
683 Capital Partners, LP	1,697,038	(4)	5.5%
Directors and Named Executive Officers			
William S. Marshall, Ph.D.	809,384	(5)	2.6%
Adam S. Levy	205,053	(6)	*
Paul D. Rubin, M.D.	196,552	(7)	*
Christopher J. Bowden, M.D.	26,666	(8)	*
Jeffrey S. Hatfield	26,666	(9)	*
Thomas E. Hughes, Ph.D.	95,939	(10)	*
Kevin Koch, Ph.D.	72,746	(11)	*
Arlene M. Morris	20,000	(12)	*
Joseph L. Turner	58,666	(13)	*
All directors and officers as a group (10 persons)	1,699,112	(14)	5.3%

* Represents beneficial ownership of less than 1% of class.

(1) Based solely upon a Schedule 13D filed with the SEC on February 23, 2017. Atlas Venture Associates VII, L.P. is the sole general partner of Atlas Venture Fund VII, L.P., and Atlas Venture Associates VII, Inc. is the sole general partner of Atlas Venture Associates VII, L.P. Atlas Venture Associates VII, L.P., by virtue of its position as the general partner of Atlas

Venture Fund VII, L.P., and Atlas Venture Associates VII, Inc., by virtue of its position as the general partner of Atlas Venture Associates VII, L.P., each may be deemed to beneficially own and share voting and dispositive power with respect to the shares of common stock owned by Atlas Venture Fund VII, L.P. The principal business address of Atlas Venture VII, L.P. is 25 First Street, Suite 303, Cambridge, MA 02141.

(2) Based solely upon a Schedule 13G/A filed with the SEC on November 13, 2018. Abigail P. Johnson is a Director, the Chairman and the Chief Executive Officer of FMR LLC. Members of the Johnson family, including Abigail P. Johnson, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. Neither FMR LLC nor Abigail P. Johnson has the sole power to vote or direct the voting of the shares owned directly by the various investment companies registered under the Investment Company Act ("Fidelity Funds") advised by Fidelity Management & Research Company ("FMR Co"), a wholly owned subsidiary of FMR LLC, which power resides with the Fidelity Funds' Boards of Trustees. FMR Co carries out the voting of the shares under written guidelines established by the Fidelity Funds' Boards of Trustees. The address for FMR, LLC is 245 Summer Street, Boston, MA 02110.

(3) Based solely upon a Schedule 13G filed with the SEC on February 17, 2017. Remeditex Ventures LLC shares voting and dispositive power over its shares of common stock with Malachite Trust, the majority owner of Remeditex Ventures LLC and Lyda Hill. Ms. Hill is the Trustee of the Malachite Trust. By reason of such relationships, Ms. Hill and the Malachite Trust may be deemed to beneficially own the shares of common stock owned directly by Remeditex Ventures LLC. The principal business address of Remeditex is 2727 N. Harwood Street, Suite 200, Dallas, TX 75201.

(4) Based solely upon a Schedule 13G filed with the SEC on January 3, 2019. 683 Capital Management, LLC and Ari Zweiman share voting and dispositive power over the shares of common stock owned by 683 Capital Partners, LP, and may be deemed to beneficially own the shares of common stock owned by 683 Capital Partners, LP. The principal business address of 683 Capital Partners, LP is 3 Columbus Circle, Suite 2205, New York, NY 10019.

(5) Includes 269,396 shares of common stock and 539,988 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(6) Includes 14,290 shares of common stock and 190,763 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(7) Includes 196,552 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(8) Includes 26,666 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(9) Includes 26,666 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(10) Includes 12,827 shares of common stock and 83,112 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(11) Includes 72,746 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(12) Includes 20,000 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(13) Includes 58,666 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019.

(14) Includes 329,750 shares of common stock and 1,369,362 shares of common stock issuable upon exercise of options to purchase our common stock within 60 days of March 1, 2019 held by our current directors and executive officers,

including William S. Marshall, Ph.D., Jason A. Leverone, Adam S. Levy, Paul D. Rubin, M.D., Christopher J. Bowden, M.D., Jeffrey S. Hatfield, Thomas E. Hughes, Ph.D., Kevin Koch, Ph.D., Arlene M. Morris, and Joseph L. Turner.

Securities Authorized for Issuance Under Equity Compensation Plans

As of December 31, 2018, we had two equity compensation plans in place under which shares of our common stock were authorized for issuance:

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights		Weighted average exercise price of outstanding options, warrants and rights	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))
	(a)		(b)	(c)
Equity compensation plans approved by security holders (1)	3,527,413	(2)	$ 5.76	705,317
Equity compensation plans not approved by security holders	—		$ —	—
Total	3,527,413		$ 5.76	705,317

(1) The 2016 Plan includes an "evergreen" feature, which provides that an additional number of shares will automatically be added to the shares reserved for issuance under the 2016 Plan on January 1st of each year, beginning on January 1, 2018 and ending on (and including) January 1, 2026. The number of shares added each calendar year will equal the lesser of: (i) 4% of the total number of shares of our common stock outstanding on December 31st of the preceding calendar year or (ii) a lesser number of shares determined by the board of directors.

(2) Represents outstanding options or warrants to purchase shares of common stock.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

Related-Person Transaction Policy and Procedures

In February 2017, we adopted a related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions. For purposes of our policy only, a related person transaction is a transaction, arrangement or relationship, or any series of similar transactions, arrangements, or relationships, in which we and any related person are, were or will be participants in which the amount involved exceeds the lesser of (x) $120,000 or (y) 1% of the average of our total assets at year end for the last two completed fiscal years. Transactions involving compensation for services provided to us as an employee or director are not covered by this policy. A related person is any executive officer, director, or beneficial owner of more than 5% of any class of our voting securities, including any of their immediate family members and any entity owned or controlled by such persons.

Under the policy, if a transaction has been identified as a related person transaction, including any transaction that was not a related person transaction when originally consummated or any transaction that was not initially identified as a related person transaction prior to consummation, our management must present information regarding the related person transaction to our audit committee, or, if audit committee approval would be inappropriate, to another independent body of our board of directors, for review, consideration and approval or ratification. The presentation must include a description of, among other things, the material facts, the interests, direct and indirect, of the related persons, the benefits to us of the transaction and whether the transaction is on terms that are comparable to the terms available to or from, as the case may be, an unrelated third party or to or from employees generally. Under the policy, we will collect information that we deem reasonably necessary from each director, executive officer and, to the extent feasible, significant stockholder to enable us to identify any existing or potential related-person transactions and to effectuate the terms of the policy. In addition, under our code of business conduct and ethics, our employees and directors will have an affirmative responsibility to disclose any transaction or relationship that reasonably could be expected to give rise to a conflict of interest. In considering related person transactions, our audit committee, or other independent body of our board of directors, will take into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs, and benefits to us;
- the impact on a director's independence in the event that the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

The policy requires that, in determining whether to approve, ratify or reject a related person transaction, our audit committee, or other independent body of our board of directors, must consider, in light of known circumstances, whether the transaction is in, or is not inconsistent with, our best interests and those of our stockholders, as our audit committee, or other independent body of our board of directors, determines in the good faith exercise of its discretion.

Certain Related-Person Transactions

Described below are the transactions and series of similar transactions since January 1, 2017 in which:

- the amounts involved exceeded or will exceed the lesser of (x) $120,000 or (y) 1% of the average of our total assets at year end for the last two completed fiscal years; and
- any of the directors, executive officers, holders of more than 5% of our capital stock (sometimes refer to as 5% stockholders below) or any member of their immediate family had or will have a direct or indirect material interest.

Public Offering of Common Stock

In February 2018, we entered into the Underwriting Agreement with the Underwriters relating to our Public Offering. Pursuant to the Underwriting Agreement, in February 2018 we sold 7,414,996 shares of common stock at a price of $5.50 per share, which resulted in net proceeds of approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses payable by us.

The table below sets forth the number of shares of our common stock purchased by and the purchase price for the shares of common stock for each purchaser that is a director, executive officer or 5% stockholder, and their affiliates.

Name of Purchaser	Shares of Common Stock	Purchase Price
Atlas Venture Fund X, L.P. (1)	545,454	$2,999,997
Adam Levy	9,090	$49,995

(1) The Atlas Venture Funds, together, hold more than 5% of our outstanding capital stock. Dr. Booth was a member of our board of directors and a director of Atlas Venture Associates VII, Inc. and Atlas Venture Associates X, Inc., which are affiliated with the Atlas Venture Funds.

Amendment to the Bennet S. Lebow Promissory Note

In connection with our initial public offering in 2014, Bennett S. LeBow advanced $1,000,000 to us to pay for certain offering expenses. Following the offering, this amount, along with an additional $45,000, which was advanced to pay for certain additional offering expenses, was reclassified as amounts due to related party on our consolidated balance sheet. This aggregate amount was non-interest bearing and due on demand.

On March 6, 2015, we issued the Note to Mr. Lebow, who was then a member of our board of directors and our largest stockholder. When issued, the terms of the Note provided: (i) for a principal amount of $1,105,009, which accrued interest computed on the basis of the actual number of days elapsed in a 360-day year, at a rate per annum of 8%; (ii) that at any time on or after June 30, 2015, Mr. LeBow may demand payment of the entire outstanding principal of the Note and all unpaid interest accrued thereon; and (iii) that upon the occurrence and during the continuance of any event of default by Signal under the Note, the principal balance of the Note shall accrue interest at a rate of 11%.

On October 31, 2016, prior to the execution of the Merger Agreement, we entered into the Note Amendment with Mr. LeBow. The Note Amendment (i) made the outstanding principal balance and all accrued interest on the Note, plus a premium of 11% on the outstanding balance, automatically convertible into shares our common stock immediately prior to the effective time of the Merger at a conversion price of $5.39 per share, which was the closing price of our common stock on the effective date of the Note Amendment, and (ii) modified the principal amount of the Note to $1,045,000, the original amount advanced to us as of June 17, 2014, and the interest of the Note to a rate per annum of 11% commencing on June 17, 2014, with interest computed on the basis of the actual number of days in a 360-day year. The terms of the Note Amendment were approved by our stockholders on February 10, 2017. Upon the closing of the Merger, the Note converted into 279,067 shares of our common stock.

Director and Officer Indemnification and Insurance

We have entered into indemnification agreements with each of our executive officers and directors and purchased directors' and officers' liability insurance. Our indemnification agreements and bylaws require us to indemnify our directors and officers to the fullest extent permitted under Delaware law.

Director Independence

Nasdaq's listing standards require that our board of directors consist of a majority of independent directors, as determined under the applicable rules and regulations of Nasdaq. Based upon information requested from and provided by each director concerning his or her background, employment, and affiliations, including family relationships, other than Dr. Marshall by virtue of his position as our chief executive officer, our board of directors believes that Drs. Bowden, Hughes, and Koch and Messrs. Hatfield, and Turner, and Ms. Morris each qualify as an independent director.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

Current Independent Registered Public Accounting Firm Fees

The following table sets forth the fees for professional services rendered by KPMG LLP, our independent registered public accounting firm, in connection with the audits of our annual financial statements for the years ended December 31, 2018 and 2017 and for other services rendered by KPMG LLP during those periods.

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Audit fees (1)	$287	$300
Audit-related fees (2)	—	—
Tax fees (3)	—	—
All other fees (4)	—	—
Total fees	$287	$300

(1) Audit fees consist of fees billed for professional services for audit and quarterly review of our financial statements and review of our registration statement for the Merger, and related services that are normally provided in connection with statutory and regulatory filings or engagements.

(2) Audit-related fees include services relating to accounting consultations and reviews and due diligence services.

(3) Tax fees include services relating to tax compliance, tax advice, and tax planning in the United States.

(4) All other fees include the aggregate of the fees billed for products and services provided by the principal accountant other than the products and services disclosed as audit fees, audit-related fees, and tax fees.

All fees described above were pre-approved by our audit committee. We have furnished the foregoing disclosure to KPMG LLP.

Other Auditors

BDO USA, LLP, or BDO, served as the independent registered public accounting firm for the audit of the financial statements for Miragen Therapeutics, Inc. (formerly Signal Genetics, Inc.), for the year ended December 31, 2016 and through the closing

of the Merger. On February 13, 2017, following the closing of the Merger, our audit committee approved the appointment of KPMG LLP as our independent registered public accounting firm to audit our financial statements for the year ended December 31, 2017, in place of BDO.

Pre-Approval Policies and Procedures

BDO served as our independent registered public accounting firm for the year ended December 31, 2016 and had served in that capacity since July 15, 2013. The decision to engage BDO as our independent registered public accounting firm for the year ended December 31, 2016 was approved by our audit committee. On February 13, 2017, our audit committee approved the appointment of KPMG LLP as our independent registered public accounting firm to audit our financial statements for the year ended December 31, 2017, in place of BDO.

Our audit committee considered the independence of BDO and KPMG LLP, as applicable, and whether the audit and non-audit services each provided to us are compatible with maintaining that independence. Our audit committee has adopted a set of policies governing the provision of non-audit services by our independent registered public accounting firm. Our audit committee has adopted procedures by which our audit committee must approve in advance all services provided by and fees paid to our independent registered public accounting firm. The advance approval requirement was not waived in any instance during the past year.

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PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

(a)(1) Financial Statements

The financial statements required by this item are submitted in a separate section beginning on page F-1 of this Annual Report.

(a)(2) Financial Statement Schedules

Financial statement schedules have been omitted because they are either not required, not applicable, or the information is otherwise included.

(a)(3) Exhibits

See Exhibit Index, which is incorporated herein by reference.

ITEM 16. FORM 10-K SUMMARY

None.

EXHIBIT INDEX

The exhibits listed in the Exhibit Index are required by Item 601 of Regulation S-K. Each management contract or compensatory plan or arrangement required to be filed as an exhibit to this Annual Report has been identified. The SEC file number for all items incorporated by reference herein from reports on Forms 10-K, 10-Q, and 8-K is 001-36483.

		Incorporated by Reference			
Exhibit Number	Description of Exhibit	Form	Filing Date	Number	Filed Herewith
3.1	Certificate of Incorporation of the Registrant.	10-Q	08/14/2014	3.1	
3.2	Certificate of Amendment of Certificate of Incorporation of the Registrant.	S-4	12/02/2016	3.3	
3.3	Certificate of Amendment of Certificate of Incorporation of the Registrant.	8-K	02/13/2017	3.1	
3.4	Certificate of Amendment of Certificate of Incorporation of the Registrant.	8-K	02/13/2017	3.2	
3.5	Amended and Restated Bylaws of the Registrant.	10-Q	08/15/2016	3.1	
3.6	Amendment to the Amended and Restated Bylaws of the Registrant.	8-K	02/13/2017	3.3	
3.7	Certificate of Ownership and Merger of the Registrant.	8-K	02/13/2017	3.4	
4.1	Specimen Common Stock Certificate.	S-1	03/19/2014	4.1	
4.2	Warrant to Purchase Stock between Miragen Therapeutics, Inc. and Silicon Valley Bank, dated April 30, 2015.	10-K	03/14/2019	4.2	
4.3	Warrant to Purchase Stock between Miragen Therapeutics, Inc. and Silicon Valley Bank, dated November 14, 2017.	8-K	11/15/2017	10.2	
10.1	Form of Indemnification Agreement between Registrant and each of its directors and executive officers.	S-1	03/19/2014	10.14	

10.1.1*	Form of Indemnity Agreement between the Registrant and each of its directors and executive officers.	S-4	12/02/2016	10.32
10.2*	Form of 2016 Equity Incentive Plan.	S-4	12/02/2016	10.37
10.2.1*	Form of Stock Option Grant Notice and Stock Option Agreement under 2016 Equity Incentive Plan.	S-4	12/02/2016	10.38
10.2.2*	Form of Restricted Stock Award Agreement under the 2016 Equity Incentive Plan.	10-Q	05/11/2017	10.12

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10.3*	Form of 2008 Equity Incentive Plan.	S-4	12/02/2016	10.48
10.3.1*	Form of Stock Option Grant Notice and Stock Option Agreement under the Registrant 2008 Equity Incentive Plan.	S-4	12/02/2016	10.49
10.4*	2016 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.39 to the Registrant's Registration Statement on S-4 (File No. 333-214893), as filed with the SEC on December 2, 2016).	S-4	12/02/2016	10.39
10.5*	Amended and Restated Non-Employee Director Compensation Policy.	10-Q	05/11/2017	10.13
10.8*	Employment Agreement by and between the Registrant and William S. Marshall, Ph.D., dated as of December 2, 2016.	S-4	12/02/2016	10.33
10.9*	Employment Agreement by and between the Registrant and Jason A. Leverone, dated as of December 2, 2016.	S-4	12/02/2016	10.34
10.10*	Employment Agreement by and between the Registrant and Adam S. Levy, dated as of December 2, 2016.	S-4	12/02/2016	10.35
10.11*	Employment Agreement by and between the Registrant and Paul D. Rubin, M.D., dated as of December 2, 2016.	S-4	12/02/2016	10.36
10.12	Lease by and between Registrant and Crestview, LLC, dated as of December 16, 2010.	S-4	12/02/2016	10.40
10.12.1	First Addendum to Lease by and between Registrant and Crestview, LLC, dated as of February 18, 2015.	S-4	12/02/2016	10.40.1
10.12.2	Second Addendum to Lease by and between Registrant and Crestview, LLC, dated as of October 23, 2015.	S-4	12/02/2016	10.40.2
10.13^	Exclusive Patent License Agreement, dated as of April 16, 2008, by and between Registrant and Board of Regents of The University of Texas System.	S-4	12/02/2016	10.41
10.14^	Exclusive Patent License Agreement, dated as of April 16, 2008, by and between Registrant and Board of Regents of The University of Texas System.	S-4	12/02/2016	10.42
10.15^	License and Collaboration Agreement, dated as of October 20, 2010, by and between Registrant and T2Cure GmbH.	S-4	12/02/2016	10.43
10.15.1	Amendment No. 1 to License and Collaboration Agreement, dated as of July 8, 2014, by and between Registrant and T2cure GmbH.	S-4	12/02/2016	10.43.1
10.16^	Amended and Restated License Agreement, dated as of December 31, 2012, by and between Registrant and Santaris Pharma A/S.	S-4	12/02/2016	10.44
10.17^	License and Collaboration Agreement, dated as of October 12, 2011, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	S-4	12/02/2016	10.45
10.17.1^	First Amendment of the License and Collaboration Agreement, effective as of May 13, 2013, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	S-4	12/02/2016	10.45.1
10.17.2^	Second Amendment of the License and Collaboration Agreement, effective as of April 10, 2014, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	S-4	12/02/2016	10.45.2
10.17.3^	Third Amendment of the License and Collaboration Agreement, effective as of May 28, 2015, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	S-4	12/02/2016	10.45.3

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10.17.4	Fourth Amendment of the License and Collaboration Agreement, effective as of September 22, 2016, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	S-4	12/02/2016	10.45.4
10.17.5^	Fifth Amendment of the License and Collaboration Agreement, effective as May 2, 2017, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	10-Q	08/11/2017	10.1
10.17.6^	Sixth Amendment of the License and Collaboration Agreement, effective as September 27, 2017, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	10-Q	11/09/2017	10.1
10.17.7^	Seventh Amendment of the License and Collaboration Agreement, entered into as of April 3, 2018 and effective as of March 26, 2018, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.	10-K	05/09/2018	10.1
10.17.8	Eighth Amendment of the License and Collaboration Agreement, entered into as of January 21, 2019 and effective as of January 7, 2019, by and between Registrant and Les Laboratoires Servier, on the first part, and Institut de Recherches Servier, on the second part.			
10.18^	Exclusive Patent License Agreement, dated as of May 10, 2016, by and between Registrant and The Brigham and Women's Hospital, Inc.	S-4	12/02/2016	10.46
10.19^	Research Subaward Agreement, dated as of October 1, 2016, by and between Registrant and Yale University, as amended.	S-4	12/02/2016	10.51
10.19.1^	Amendment to Research Subaward Agreement, effective as of October 27, 2016, by and between Registrant and Yale University.	10-Q/A	06/07/2017	10.14
10.19.2^	Amendment to Research Subaward Agreement, effective as of July 1, 2017, by and between Registrant and Yale University.	10-Q	11/09/2017	10.2
10.19.3^	Amendment to Research Subaward Agreement, entered into as of May 30, 2018 and effective as of May 17, 2018, by and between Registrant and Yale University, as amended.	10-Q	08/08/2018	10.2
10.19.4^	Amendment to Research Subaward Agreement, entered into as of June 29, 2018 and effective as of July 1, 2018, by and between Registrant and Yale University, as amended.	10-Q	08/08/2018	10.3
10.20	Loan and Security Agreement, dated as of April 30, 2015, by and between the Registrant and Silicon Valley Bank.	S-4	12/02/2016	10.47
10.20.1	First Loan Modification Agreement, dated as of December 22, 2016, by and between the Registrant and Silicon Valley Bank.	S-4	01/04/2017	10.47.1
10.21	Amended and Restated Loan and Security Agreement between Miragen Therapeutics, Inc. and Silicon Valley Bank, dated November 14, 2017.	8-K	11/15/2017	10.1
10.22	Common Stock Sales Agreement, dated March 31, 2017, by and between the Registrant and Cowen and Company, LLC.	8-K	03/31/2017	10.1
10.23^	Common Stock Purchase Agreement, dated August 6, 2018, by and between the Registrant and The Leukemia & Lymphoma Society, Inc.	10-Q	11/07/2018	10.2
21.1	Subsidiaries of the Registrant.			

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23.1	Consent of Independent Registered Public Accounting Firm.
24.1	Power of Attorney (included on signature page hereto).
31.1	Certification of Principal Executive Officer pursuant to Rule13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act, as amended.
31.2	Certification of Principal Financial Officer pursuant to Rule13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act, as amended.
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. 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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^ Confidential treatment has been granted as to portions of the exhibit. Confidential materials omitted and filed separately with the SEC.

* This certification is being furnished pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof.

** In accordance with Rule 406T of Regulation S-T, the Interactive Data Files in Exhibit 101 are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of Section 18 of the Exchange Act of 1934, as amended, and otherwise are not subject to liability under these sections.

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SIGNATURES

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

MIRAGEN THERAPEUTICS, INC.

Date: March 14, 2019 By: /s/ William S. Marshall
William S. Marshall, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Date: March 14, 2019 By: /s/ Jason A. Leverone
Jason A. Leverone
Chief Financial Officer
(Principal Financial Officer; Principal Accounting Officer)

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KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints William S. Marshall and Jason A. Leverone, and each of them, as his or her attorneys-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to this Annual Report on Form 10-K, and to file the same, with exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that said attorneys-in-fact, and each of them, or his substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of l934, 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/ William S. Marshall, Ph.D. William S. Marshall, Ph.D.	Chief Executive Officer and Director (Principal Executive Officer)	March 14, 2019
/s/ Jason A. Leverone Jason A. Leverone	Chief Financial Officer, Treasurer, and Secretary (Principal Financial Officer; Principal Accounting Officer)	March 14, 2019
/s/ Jeffrey S. Hatfield Jeffrey S. Hatfield	Chairman of the Board	March 14, 2019
/s/ Christopher Bowden, M.D. Christopher Bowden, M.D.	Director	March 14, 2019
/s/ Thomas E. Hughes, Ph.D. Thomas E. Hughes, Ph.D.	Director	March 14, 2019
/s/ Arlene M. Morris Arlene M. Morris	Director	March 14, 2019
/s/ Kevin Koch, Ph.D. Kevin Koch, Ph.D.	Director	March 14, 2019
/s/ Joseph L. Turner Joseph L. Turner	Director	March 14, 2019

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MIRAGEN THERAPEUTICS, INC.
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Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
Miragen Therapeutics, Inc.:

Opinion on the Consolidated Financial Statements
We have audited the accompanying consolidated balance sheets of Miragen Therapeutics, Inc. and subsidiary (the Company) as of December 31, 2018 and 2017, the related consolidated statements of operations and comprehensive loss, changes in preferred stock and stockholders' equity (deficit), and cash flows for the years then ended, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.
Basis for Opinion
These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.
We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. 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 consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ KPMG LLP

We have served as the Company's auditor since 2009.
Denver, Colorado
March 14, 2019

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MIRAGEN THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)

	December 31,	
	2018	2017
Assets		
Current assets:		
Cash and cash equivalents	$32,606	$47,441
Short-term investments	29,875	—
Accounts receivable	24	1,456
Prepaid expenses and other current assets	2,865	2,971
Total current assets	65,370	51,868
Property and equipment, net	727	563
Other assets	50	50
Total assets	$66,147	$52,481
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$571	$906
Accrued liabilities	3,868	2,991
Current portion of note payable	2,294	—
Total current liabilities	6,733	3,897
Note payable, net of current portion	8,004	9,922
Other liabilities	66	152
Total liabilities	14,803	13,971
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value; 100,000,000 shares authorized; 30,839,463 and 22,568,006 shares issued and outstanding at December 31, 2018 and 2017, respectively	308	226
Additional paid-in capital	177,335	131,877
Accumulated other comprehensive loss	(3)	—
Accumulated deficit	(126,296)	(93,593)
Total stockholders' equity	51,344	38,510
Total liabilities and stockholders' equity	$66,147	$52,481

See accompanying notes to these consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)

	Year Ended December 31,	
	2018	2017
Revenue:		
Collaboration revenue	$7,373	$3,097
Grant revenue	1,013	906
Total revenue	8,386	4,003
Operating expenses:		
Research and development	30,421	19,623
General and administrative	11,049	10,912
Total operating expenses	41,470	30,535
Loss from operations	(33,084)	(26,532)
Other income (expense):		
Interest and other income	1,254	403
Interest and other expense	(873)	(383)
Net loss	(32,703)	(26,512)
Change in unrealized loss on investments	(3)	—
Comprehensive loss	$(32,706)	$(26,512)
Net loss	$(32,703)	$(26,512)
Accretion of redeemable convertible preferred stock to redemption value	—	(5)
Net loss available to common stockholders	$(32,703)	$(26,517)
Net loss per share, basic and diluted	$(1.10)	$(1.38)
Weighted-average shares used to compute basic and diluted net loss per share	29,600,332	19,244,605

See accompanying notes to these consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC
CONSOLIDATED STATEMENTS OF CHANGES IN PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
(in thousands, except share data)

	Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balance at December 31, 2016	18,584,390	$76,976	833,744	$ 8	$5,147	$ —	$(67,076)	$(61,921)
Issuance of common stock, net of issuance cost; private financing	—	—	6,359,628	64	39,092	—	—	39,156
Issuance of common stock, net of issuance cost; at the market	—	—	840,534	8	7,595	—	—	7,603
Accretion of redeemable convertible preferred stock to redemption value	—	5	—	—	—	—	(5)	(5)
Conversion of preferred stock to common stock	(18,584,390)	(76,981)	13,066,666	131	76,850	—	—	76,981
Issuance of common stock upon reverse merger	—	—	1,024,960	10	194	—	—	204
Reclassification of warrant liability to equity	—	—	—	—	51	—	—	51
Issuance of common stock upon cashless exercise of warrant	—	—	16,387	—	—	—	—	—
Exercises of stock options and issuance of restricted stock awards	—	—	412,894	5	302	—	—	307
Issuance of common stock for cash under employee stock purchase plan	—	—	13,193	—	110	—	—	110
Issuance of warrant classified as equity	—	—	—	—	127	—		127
Share-based compensation expense	—	—	—	—	2,409	—	—	2,409
Net loss	—	—	—	—	—	—	(26,512)	(26,512)
Balance at December 31, 2017	—	—	22,568,006	226	131,877	—	(93,593)	38,510
Issuance of common stock in public offering, net of issuance cost	—	—	7,414,996	74	37,771	—	—	37,845
Issuance of common stock, net of issuance cost; at the market	—	—	372,852	4	2,653		—	2,657

Issuance of common stock in private placement, net of issuance costs	—	—	150,987	2	933	—	—	935
Shares issued for cash upon the exercise of stock options	—	—	280,178	2	180	—	—	182
Issuance of common stock for cash under employee stock purchase plan	—	—	52,444	—	242	—	—	242
Share-based compensation expense	—	—	—	—	3,679	—	—	3,679
Change in unrealized loss on investments	—	—	—	—	—	(3)	—	(3)
Net loss	—	—	—	—	—	—	(32,703)	(32,703)
Balance at December 31, 2018	—	$—	30,839,463	$ 308	$177,335	$ (3)	$(126,296)	$51,344

See accompanying notes to these consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	
	2018	2017
Cash flows from operating activities:		
Net loss	$(32,703)	$(26,512)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation expense	3,679	2,409
Non-cash interest expense	376	94
Depreciation and amortization	281	308
Amortization of premiums and discounts on available-for-sale securities	(416)	—
Changes in operating assets and liabilities:		
Accounts receivable	1,432	(1,436)
Prepaid expenses and other assets	51	(724)
Accounts payable	(335)	(101)
Accrued and other liabilities	791	(2,205)
Net cash used in operating activities	(26,844)	(28,167)
Cash flows from investing activities:		
Purchases of short-term investments	(62,462)	—
Maturities of short-term investments	33,000	—
Purchases of property and equipment	(445)	(246)
Cash acquired in reverse merger	—	1,280
Proceeds from sale of property and equipment	—	—
Net cash provided by (used in) investing activities	(29,907)	1,034
Cash flows from financing activities:		
Proceeds from the sale of common stock - public offering	40,782	—
Payment of issuance costs associated with the sale of common stock - public offering	(2,890)	—
Proceeds from the sale of common stock - at the market	2,747	7,862
Payment of issuance costs associated with the sale of common stock - at the market	(82)	(330)
Proceeds from the sale of common stock - private financing	1,000	40,703
Payment of issuance costs associated with the sale of common stock - private placement	(65)	(1,216)
Payment of issuance costs associated with the shelf registration	—	(299)
Proceeds from stock purchases under employee stock purchase plan	242	110
Proceeds from the exercise of stock options	182	307
Proceeds from issuance of notes payable	—	10,000
Payments of principal on note payable	—	(4,667)
Net cash provided by financing activities	41,916	52,470
Net increase (decrease) in cash and cash equivalents	(14,835)	25,337
Cash and cash equivalents at beginning of period	47,441	22,104
Cash and cash equivalents at end of period	$32,606	$47,441

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MIRAGEN THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS (Continued)
(in thousands)

	Year Ended December 31,	
	2018	2017
Supplemental disclosure of cash flow information		
Cash paid for interest	$489	$446
Supplemental disclosure of non-cash investing and financing activities		
Amortization of public offering costs	$55	$—
Change in unrealized loss on investments	$(3)	$—
Conversion of preferred stock to common stock	$—	$76,981
Liabilities assumed, net of non-cash assets received in reverse merger	$—	$1,076
Transfer of common stock issuance costs from prepaid expenses and other current assets to equity (private financing and at the market sales)	$—	$331
Transfer of common stock issuance costs from prepaid expenses and other current assets to equity (at the market / shelf costs)	$—	$23
Issuance of warrant classified as equity	$—	$127
Reclassification of preferred stock warrant (accrued liability) to common stock warrant (equity)	$—	$51
Accretion of redeemable convertible preferred stock to redemption value	$—	$5

See accompanying notes to these consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. DESCRIPTION OF BUSINESS

Miragen Therapeutics, Inc., a Delaware corporation (the "Company" or "Miragen"), is a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in certain diseases where there is a high unmet medical need. microRNAs are short RNA molecules, or oligonucleotides, that regulate gene expression and play vital roles in influencing the pathways responsible for many disease processes. The Company has three product candidates, cobomarsen, remlarsen, and MRG-110 in clinical development. The Company is independently developing cobomarsen and remlarsen and is developing MRG-110 under a license and collaboration agreement (the "Servier Collaboration Agreement") with Les Laboratoires Servier and Institut de Recherches Servier (collectively, "Servier").

Liquidity

The Company has incurred annual net operating losses since its inception. As of December 31, 2018, the Company had an accumulated deficit of $126.3 million and a net loss of $32.7 million and $26.5 million for the years ended December 31, 2018 and 2017, respectively. The Company's management believes that the $32.6 million of cash and cash equivalents and $29.9 million of short-term investments on hand at December 31, 2018 will be sufficient to fund its operations in the normal course of business and allow the Company to meet its liquidity needs through the first quarter of 2020.

The Company has funded its operations to date principally through proceeds from equity financings, including notes payable that previously converted to equity explained further in Note 9. Common Stock. The Company will continue to require additional capital beyond the first quarter of 2020 to continue its operations. The amount and timing of future funding requirements will depend on many factors, including the pace and results of the Company's clinical development efforts, continued performance under the Servier Collaboration Agreement, securing additional partnerships and collaborations, and issuing debt or other financing vehicles. The Company's ability to secure additional capital is dependent upon a number of factors, some of which are outside of the Company's control, including success in developing its technology and drug product candidates, operational performance, and market conditions.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiary, Miragen Therapeutics Europe Limited, which was formed in January 2011 and has no employees or operations. In February 2019, the Company created a new wholly-owned subsidiary, miRagen Therapeutics S.à.r.l, which also has no employees or operations. Both subsidiaries were formed for the sole purpose of submitting regulatory filings in Europe.

The consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position, results of operations, and cash flows for the periods presented. All significant intercompany balances have been eliminated in consolidation. The Company's management performed an evaluation of its activities through the date of filing of these financial statements and concluded that there are no subsequent events requiring disclosure, other than as disclosed.

Use of Estimates

The Company's consolidated financial statements are prepared in accordance with U.S. GAAP, which requires it to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and contingent liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Although these estimates are based on the Company's knowledge of current events and actions it may take in the future, actual results may ultimately differ from these estimates and assumptions.

Revenue Recognition

The Company recognizes revenue principally from its strategic alliance and collaboration agreements. Revenue is recognized from upfront payments for licenses and milestone payments that are generated from defined research or development events, as well as from the reimbursement of amounts for research and development services under its strategic alliance and collaboration agreement. The Company recognizes revenue when all four of the following criteria are met: (i) persuasive evidence of an

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arrangement exists; (ii) products have been delivered or services rendered; (iii) the selling price is fixed or determinable; and (iv) collectability is reasonably assured.

Multiple-element arrangements are examined to determine whether the deliverables can be separated or must be accounted for as a single unit of accounting. The Servier Collaboration Agreement, for example, includes a combination of upfront license fees, payments for research and development activities, and milestone payments that are evaluated to determine whether each deliverable under the agreement has value to the customer on a stand-alone basis and whether reliable evidence of fair value for the deliverable exists. Deliverables in an arrangement that do not meet this separation criteria are treated as a single unit of accounting, generally applying applicable revenue recognition guidance for the final deliverable to the combined unit of accounting.

The Company recognizes revenue from non-refundable upfront license fees over the term of performance when combined with other deliverables under the applicable agreement. When the performance period is not specified, the Company estimates the performance period based upon provisions contained within the agreement, such as the duration of the research or development term, the existence, or likelihood, of achievement of development commitments, and any other significant commitments. These advance payments are deferred and recorded as deferred revenue upon receipt, pending recognition, and are classified as a short-term or long-term liability in the accompanying consolidated balance sheets. Expected performance periods are reviewed periodically and, if applicable, the amortization period is adjusted, which may accelerate or decelerate revenue recognition. The timing of revenue recognition, specifically as it relates to the amortization of upfront license fees, is significantly influenced by the Company's estimates.

The Company applies the milestone method of accounting to recognize revenue from milestone payments when earned, as evidenced by persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. A milestone event is defined as an event (i) that can only be achieved based in whole or in part on either the Company's performance or on the occurrence of a specific outcome resulting from the Company's performance; (ii) for which there is substantive uncertainty at the inception of the arrangement that the event will be achieved; and (iii) that would result in additional payments being due to the Company. Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty's performance are not considered to be milestone events. A milestone event is substantive if all of the following conditions are met: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value to the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone; (ii) the consideration relates solely to past performance; and (iii) the consideration is reasonable relative to all the deliverables and payment terms (including other potential milestone consideration) within the arrangement. The Company assesses whether a milestone is substantive at the inception of each arrangement. If a milestone is deemed non-substantive, the Company accounts for the milestone payment using a method consistent with the related units of accounting for the arrangement over the estimated performance period.

Share-Based Compensation

The Company accounts for share-based compensation expense related to stock options granted to employees and members of its board of directors under its 2008 Equity Incentive Plan (the "2008 Plan") and under its 2016 Equity Incentive Plan (the "2016 Plan") by estimating the fair value of each stock option or award on the date of grant using the Black-Scholes option pricing model. The Company recognizes share-based compensation expense on a straight-line basis over the vesting term.

The Company accounts for stock options issued to non-employees (other than board members) by valuing the award using the Black-Scholes option pricing model and remeasuring such awards to the current fair value until the awards are vested or a performance commitment has otherwise been reached.

Research and Development

Research and development costs are expensed as incurred in performing research and development activities. The costs include employee-related expense including salaries, benefits, share-based compensation, fees for acquiring and maintaining licenses under third-party license agreements, consulting fees, market research, costs of research and development activities conducted by third parties on the Company's behalf, costs to manufacture or have manufactured clinical trial materials, laboratory supplies, depreciation, and facilities and overhead costs. The Company records research and development expense in the period in which the Company receives or takes ownership of the applicable goods or when the applicable services are performed. In circumstances where amounts have been paid in excess of costs incurred, the Company records a prepaid expense.

The Company records upfront and milestone payments to acquire and retain contractual rights to licensed technology as research and development expenses when incurred if there is uncertainty in the Company receiving future economic benefit

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from the acquired contractual rights. The Company considers future economic benefits from acquired contractual rights to licensed technology to be uncertain until such a drug candidate is approved for sale by the U.S. Food and Drug Administration or when other significant risk factors are abated.

Clinical Trial and Preclinical Study Accruals

The Company makes estimates of accrued expenses as of each balance sheet date in its consolidated financial statements based on certain facts and circumstances at that time. The Company's accrued expenses for clinical trials and preclinical studies are based on estimates of costs incurred for services provided by clinical research organizations, manufacturing organizations, and other providers. Payments under the Company's agreements with external service providers depend on a number of factors, such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, the Company obtains information from various sources and estimates the level of effort or expense allocated to each period. Adjustments to the Company's research and development expenses may be necessary in future periods as its estimates change.

Cash and Cash Equivalents

All highly-liquid investments that have maturities of 90 days or less at the date of purchase are classified as cash equivalents. Cash equivalents are reported at cost, which approximates fair value due to the short maturities of these instruments.

Investments

The Company has designated its investments as available-for-sale securities and accounts for them at their respective fair values. The securities are classified as short-term or long-term based on the nature of the securities and their availability to meet current operating requirements. Securities that are readily available for use in current operations are classified as short-term available-for-sale securities and are reported as a component of current assets in the accompanying consolidated balance sheets.

Securities that are classified as available-for-sale are measured at fair value, including accrued interest, with temporary unrealized gains and losses reported as a component of stockholders' equity until their disposition. The Company reviews available-for-sale securities at the end of each period to determine whether they remain available-for-sale based on its then current intent. The cost of securities sold is based on the specific identification method.

The securities are subject to a periodic impairment review. An impairment charge would occur when a decline in the fair value of the investments below the cost basis is judged to be other-than-temporary.

As of December 31, 2018, the Company's short-term available-for-sale securities had an amortized cost of $29.9 million, fair value of $29.9 million, and a gross unrealized loss of $3 thousand. The Company had no long-term investments as of December 31, 2018. The Company had no investments as of December 31, 2017.

Fair Value Measurements

The following tables present information about the Company's financial assets and liabilities that have been measured at fair value and indicate the fair value of the hierarchy of the valuation inputs utilized to determine such fair value. In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities. Fair value determined by Level 2 inputs utilize observable inputs other than Level 1 prices, such as quoted prices, for similar assets or liabilities, quoted market prices in markets that are not active, or other inputs that

are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities. Fair values determined by Level 3 inputs are unobservable data points for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability.

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	December 31, 2018		December 31, 2017	
	Level 1	Level 3	Level 1	Level 3
	(in thousands)			
Assets:				
Money market funds (included in cash and cash equivalents) (1)	$32,936	$ —	$47,653	$ —
U.S. treasury securities (included in short-term investments)	29,875	—	—	—
Total assets	$62,811	$ —	$47,653	$ —
Liabilities:				
Common stock warrants (included in accrued and other liabilities)	$—	$ 82	$—	$ 82

(1) The sum of amounts presented for each period above differ from cash and cash equivalents reported in the consolidated balance sheets due to uninvested cash balances and outstanding disbursements and deposits.

Fair Value of Financial Instruments

Certain of the Company's financial instruments are not measured at fair value on a recurring basis but are recorded at amounts that approximate their fair value due to the short-term nature of their maturities, such as cash and cash equivalents, accounts receivable, accounts payable, and accrued expenses. The carrying amount of the Company's note payable approximates its fair value (a Level 2 fair value measurement), reflecting interest rates currently available to the Company.

The Company accounts for warrants to purchase its stock pursuant to ASC Topic 470, Debt, and ASC Topic 480, Distinguishing Liabilities from Equity, and classifies warrants for common stock as liabilities or equity. The warrants classified as liabilities are reported at their estimated fair value and any changes in fair value are reflected in interest expense and other related expenses. The warrants classified as equity are reported at their estimated fair value with no subsequent remeasurement.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk consist primarily of cash equivalents, which include short-term investments that have maturities of less than three months. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts. The Company invests its excess cash primarily in deposits and money market funds held with one financial institution.

Property and Equipment

The Company carries its property and equipment at cost, less accumulated depreciation and amortization. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, generally three to five years. Leasehold improvements are amortized over the shorter of the life of the lease (including any renewal periods that are deemed to be reasonably assured) or the estimated useful life of the assets. Construction in progress is not depreciated until placed in service. Repairs and maintenance costs are expensed as incurred and expenditures for major improvements are capitalized.

Impairment of Long-Lived Assets

The Company assesses the carrying amount of its property and equipment whenever events or changes in circumstances indicate the carrying amount of such assets may not be recoverable. No impairment charges were recorded during the years ended December 31, 2018 and 2017.

Net Loss per Share

Basic net loss per share is calculated by dividing the net loss applicable to common stockholders by the weighted average number of shares of Common Stock outstanding during the period without consideration of Common Stock equivalents. Since the Company was in a loss position for all periods presented, diluted net loss per share is the same as basic net loss per share for all periods, as the inclusion of all potential common shares outstanding is anti-dilutive.

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Comprehensive Loss

Comprehensive loss is comprised of net loss and adjustments for the change in unrealized gains and losses on investments. Unrealized accumulated comprehensive gains or losses are reflected as a separate component in the statement of stockholders' equity. As of December 31, 2018, the Company had accumulated other comprehensive loss of $3 thousand. The Company had no accumulated other comprehensive income (loss) at December 31, 2017, and no realized gain or loss during the years ended December 31, 2018 and 2017.

Income Taxes

The Company accounts for income taxes by using an asset and liability method of accounting for deferred income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. A valuation allowance is recorded to the extent it is more likely than not that a deferred tax asset will not be realized. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in operations in the period that includes the enactment date.

The Company has no unrecognized tax benefits. The Company classifies interest and penalties arising from the underpayment of income taxes in the consolidated statements of operations and comprehensive loss as general and administrative expenses. No such expenses have been recognized during the years ended December 31, 2018 and 2017.

The Tax Cuts and Jobs Act of 2017 ("Tax Act") was signed into law on December 22, 2017. The Tax Act includes significant changes to the U.S. corporate income tax system, including: (i) a federal corporate rate reduction from 35% to 21%; (ii) limitations on the deductibility of interest expense and executive compensation; (iii) elimination of the corporate alternative minimum tax ("AMT") and a change in how existing AMT credits can be realized; (iv) change in the rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017; (v) reduction of the orphan drug credit from 50% to 25%; and (vi) transition of U.S. international taxation from a worldwide tax system to a territorial tax system. The Tax Act did not have a material impact on the consolidated financial statements primarily due to the valuation allowance recorded against its net deferred tax assets.

Segment Information

The Company operates in one operating segment and, accordingly, no segment disclosures have been presented herein. All equipment, leasehold improvements, and other fixed assets are physically located within the United States and all agreements with the Company's partners are denominated in U.S. dollars, except where noted.

Recent Accounting Pronouncements – Not Yet Adopted

Revenue Recognition

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-09, Revenue from Contracts with Customers (Accounting Standards Codification Topic 606) ("ASC 606"), and has issued a number of clarifying ASUs subsequently, all of which outline a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The core principle of the revenue model is that "an entity recognizes revenue to depict the transfer of promised goods or services to customers in an amount that reflects the

consideration to which the entity expects to be entitled in exchange for those goods or services." The standard provides enhancements to the quality and consistency of how revenue is reported by companies, while also improving comparability in the financial statements of companies reporting using International Financial Reporting Standards or U.S. GAAP. The new standard also will require enhanced revenue disclosures, provide guidance for transactions that were not previously addressed comprehensively, and improve guidance for multiple-element arrangements. This accounting standard becomes effective for the Company for reporting periods beginning after December 15, 2018, and interim reporting periods thereafter. Early adoption is permitted for annual reporting periods (including interim periods) beginning after December 15, 2016. This new standard permits the use of either the retrospective or cumulative effect transition method.

The Company has assessed the impact of ASC 606 on its accounting policies and procedures and has evaluated the new requirements as applied to existing revenue contracts. Based on this assessment, the Company does not believe the adoption of

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ASC 606 will have a material impact on its consolidated financial statements. The Company did not elect early adoption of ASC 606 and will implement any changes as required by the adoption of ASC 606 beginning in the first quarter of 2019, using a modified retrospective method of adoption.

Leases

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842) and subsequent amendments to the initial guidance: ASU No. 2017-13, ASU No. 2018-10, and ASU No. 2018-11 (collectively, "Topic 842"). Topic 842 requires companies to generally recognize on the balance sheet operating and financing lease liabilities and corresponding right-of-use assets. The standard is effective for the Company for interim and annual reports beginning after December 15, 2019, with early adoption permitted. At adoption, this update will be applied using a modified retrospective approach, with an option to use certain transition relief. The Company currently expects that its building operating lease commitments will be subject to the new standard and recognized as operating lease liabilities and right-of-use assets upon its adoption of Topic 842, which will increase the Company's total assets and total liabilities that are reported relative to such amounts prior to adoption. The Company is continuing to evaluate the full impact of this standard on its consolidated financial statements.

Share-based Compensation

In June 2018, the FASB issued ASU No. 2018-07, Compensation—Stock Compensation (Topic 718): Improvements to Non-employee Share-Based Payment Accounting, which simplifies the accounting for share-based payments to non-employees by aligning it with the accounting for share-based payments to employees, with specified exceptions. This standard is effective for the Company in the first quarter of 2020, and early adoption is permitted. The Company expects the adoption of this standard will not have a significant impact on its consolidated financial statements upon adoption.

Fair Value Measurement

In August 2018, the FASB issued ASU No. 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement, which modifies the disclosure requirements of fair value measurements. This standard is effective for the Company in the first quarter of 2020, and early adoption is permitted. The Company is currently evaluating the impact of the pending adoption of this standard on its consolidated financial statements.

3. STRATEGIC ALLIANCE AND COLLABORATION WITH SERVIER

Collaboration revenue under the Servier Collaboration Agreement consisted of the following:

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Milestone payments	$3,690	$—
Research and development reimbursable costs	3,683	3,097
Total collaboration revenue	$7,373	$3,097

In October 2011, the Company entered into the Servier Collaboration Agreement with Servier for the research, development, and commercialization of RNA-targeting therapeutics in cardiovascular disease. Under the Servier Collaboration Agreement, the Company granted Servier an exclusive license to research, develop, manufacture, and commercialize RNA-targeting therapeutics for certain microRNA targets in the cardiovascular field. In 2017, the

Company and Servier agreed to amend the Servier Collaboration Agreement to remove all existing targets, add one new target (microRNA-92), and grant Servier the right to add one additional target through September 2019.

In April 2018, the Company and Servier entered into a seventh amendment to the Servier Collaboration Agreement (the "Servier Amendment"). The Servier Amendment, among other things, (i) updated the development plan for MRG-110 and cost-sharing provisions; (ii) provided for specified development cost reimbursement by Servier to the Company following a determination by a joint committee established by the parties under the Servier Collaboration Agreement that the outcome of a specified portion of a Phase 1 clinical trial has met its primary end point; and (iii) provided for additional development plan cost reimbursement by Servier to the Company following a determination by a joint committee established by the parties under the Servier Collaboration Agreement that a product candidate targeting microRNA-92 will proceed into a Phase 2 clinical trial.

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Servier's rights to each named target are limited to therapeutics in the field of cardiovascular disease, as defined, and in their territory, which is worldwide except for the United States and Japan. The Company retains all other rights including commercialization of therapeutics developed under the Servier Collaboration Agreement in the field of cardiovascular disease in the United States and Japan.

The Company is eligible to receive non-refundable development milestone payments of €5.8 million to €13.8 million ($6.6 million to $15.8 million as of December 31, 2018) and regulatory milestone payments of €10.0 million to €40.0 million ($11.4 million to $45.8 million as of December 31, 2018) for each target. Additionally, the Company may receive up to €175.0 million ($200.3 million as of December 31, 2018) in commercialization milestones, as well as quarterly royalty payments expressed in percentages ranging from the low-double digits to the mid-teens (subject to reductions for patent expiration, generic competition, third-party royalty, and costs of goods) on the net sales of any licensed product commercialized by Servier. Servier is obligated to make royalty payments for a period specified under the Servier Collaboration Agreement.

The Company applies the milestone method of accounting to recognize revenue from milestone payments when earned under the Servier Collaboration agreement, as evidenced by persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. In March 2018, the Company and Servier initiated a Phase 1 clinical trial of MRG-110. As a result, under the terms of the Servier Collaboration Agreement, the Company earned and received its first development milestone payment of €3.0 million (or $3.7 million). This amount is included as revenue in the accompanying consolidated statements of operations and comprehensive loss during the year ended December 31, 2018.

As part of the Servier Collaboration Agreement, the Company established a multiple-year research collaboration, under which it jointly performs agreed upon research activities directed to the identification and characterization of named targets and oligonucleotides in the cardiovascular field, which is referred to as the Research Collaboration. The current amended term of the Research Collaboration extends through September 2019. Servier is responsible for funding certain costs of the Research Collaboration, as defined under the Servier Collaboration Agreement.

The development of each product candidate (commencing with registration enabling toxicology studies) under the Servier Collaboration Agreement is performed pursuant to a mutually agreed upon development plan to be conducted by the parties as necessary to generate data useful for both parties to obtain regulatory approval of such product candidates. Servier is responsible for a specified percentage of the cost of research and development activities under the development plan through the completion of one or more Phase 2 clinical trials and will reimburse the Company for a specified portion of such costs it incurs. The costs of Phase 3 clinical trials for each product candidate will be allocated between the parties at a specified percentage of costs. The applicable percentage for each product candidate will be based upon whether certain events under the Servier Collaboration Agreement occur, including if the Company enters into a third-party agreement for the development and/or commercialization of a product in the United States at least 180 days before the initiation of the first Phase 3 clinical trial, or if the Company subsequently enters into a U.S. partner agreement, or if it does not enter into a U.S. partner agreement but files for approval in the United States using data from the Phase 3 clinical trial.

Under the Servier Collaboration Agreement, the Company also granted Servier a royalty-free, non-exclusive license to develop a companion diagnostic in its territory for any therapeutic product that may be developed by Servier under the Servier Collaboration Agreement. The Company also granted Servier an exclusive, royalty-free license to commercialize such a companion diagnostic in its territory for use in connection with the development and commercialization of such therapeutic product in its territory.

The Servier Collaboration Agreement will expire as to each underlying product candidate when Servier's royalty obligations as to such product candidate have expired. Servier may also terminate the Servier Collaboration

Agreement for: (i) convenience upon a specified number of days' prior notice to the Company or (ii) upon determination of a safety issue relating to development under the agreement upon a specified number of days' prior notice to the Company. Either party may terminate the Servier Collaboration Agreement upon a material breach by the other party that is not cured within a specified number of days. The Company may also terminate the agreement if Servier challenges any of the patents licensed by the Company to Servier.

The Company determined that the elements within the Servier Collaboration Agreement should be treated as a single unit of accounting because the delivered elements, the licenses, did not have stand-alone value to Servier at the time the license was granted. As such, the Company recognized license fees earned under the Servier Collaboration Agreement as revenue on a proportional performance basis over the estimated period to complete the activities under the Research Collaboration. The total period of performance is equal to the estimated term of the Research Collaboration. The Company measured its progress under the proportional performance method based on actual and estimated full-time equivalents. The Company received a total of $12.4 million (€9.0 million) in non-refundable license fees under the Servier Collaboration Agreement. Based on earlier

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estimates of the term of the Research Collaboration, these license fees had been fully recognized as revenue during the period from October 2011 through December 2016. Accordingly, no amounts were recognized as license revenue during the years ended December 31, 2018 and 2017.

Amounts incurred but not billed to Servier for research and related intellectual property activities totaled $0.5 million and $1.1 million as of December 31, 2018 and December 31, 2017, respectively. These amounts are included in prepaid expenses and other current assets in the Company's consolidated balance sheets. As of December 31, 2018, the company had no accounts receivable outstanding for Servier research and related intellectual property activities. As of December 31, 2017, accounts receivable for Servier research and related intellectual property activities totaled $1.4 million.

4. REVERSE MERGER

In February 2017, the Company, then known as Signal Genetics, Inc. ("Signal"), completed its merger with Miragen Therapeutics, Inc., a then privately-held Delaware corporation ("Private Miragen"). Pursuant to the Agreement and Plan of Merger and Reorganization (the "Merger Agreement") by and among the Company, Private Miragen, and Signal Merger Sub, Inc., a wholly-owned subsidiary of the Company ("Merger Sub"), Merger Sub merged with and into Private Miragen, with Private Miragen surviving as a wholly-owned subsidiary of the Company (the "Merger"). Immediately, following the Merger, Private Miragen merged with and into the Company, with the Company as the surviving corporation (the "Short-Form Merger" and, together with the Merger, the "Mergers"). In connection with the Short-Form Merger, the Company changed its corporate name to "Miragen Therapeutics, Inc."

For accounting purposes, Private Miragen is considered to have acquired Signal in the Merger. Private Miragen was determined to be the accounting acquirer based upon the terms of the Merger and other factors including: (i) the Private Miragen security holders owned approximately 95.2% of the combined company's outstanding common stock immediately following the closing of the Mergers; (ii) former Private Miragen directors held all of the board seats in the combined company immediately following the closing of the Mergers; and (iii) Private Miragen management held key management positions of the combined company. The Merger has been accounted for as an asset acquisition rather than business combination because the assets acquired and liabilities assumed by Private Miragen do not meet the definition of a business as defined by U.S. GAAP. The net assets acquired in connection with this transaction were recorded at their estimated acquisition date fair values in February 2017 as of the date the Mergers were completed.

Immediately prior to the effective date of the Merger, all shares of preferred stock of Private Miragen converted into shares of common stock of Private Miragen on a one-for-one basis.

At the effective date of the Merger, the Company issued shares of its Common Stock to Private Miragen stockholders, at an exchange rate of approximately 0.7031 shares of Common Stock in exchange for each share of Private Miragen common stock outstanding immediately prior to the Merger. The exchange rate was calculated by a formula that was determined through arms-length negotiations between the Company and Private Miragen. The combined company assumed all the outstanding options, whether or not vested, under the 2008 Plan with such options representing the right to purchase a number of shares of Common Stock equal to approximately 0.7031 multiplied by the number of shares of Private Miragen common stock previously represented by such options.

Immediately after the Merger, there were 21,309,440 shares of Common Stock outstanding. In addition, immediately after the Merger, Private Miragen stockholders, warrant holders, and option holders owned approximately 95.9% of the aggregate number of shares of Common Stock, and the stockholders of the Company immediately prior to the Merger owned approximately 4.1% of the aggregate number of shares of Common Stock (each on a fully diluted basis). The accompanying consolidated financial statements and notes to the consolidated financial statements give retroactive effect to the exchange ratio and change in par value for all periods presented.

On February 13, 2017, prior to the effectiveness of the Merger, Signal had 1,024,960 shares of Common Stock outstanding and a market capitalization of $12.6 million. The estimated fair value of the net assets of Signal on February 13, 2017, prior to the Merger, was $0.2 million. The fair value of Common Stock on the Merger closing date, prior to the Merger, was above the fair value of the Company's net assets. As the Company's net assets were predominantly comprised of cash offset by current liabilities, the fair value of the Company's net assets as of February 13, 2017, prior to the Merger, is considered to be the best indicator of the fair value and, therefore, the purchase consideration.

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The following table summarizes the net assets acquired based on their estimated fair values immediately prior to the Merger (in thousands):

Cash and cash equivalents	$1,280
Prepaid and other assets	248
Accrued liabilities	(1,324)
Net acquired tangible assets	$204

5. PROPERTY AND EQUIPMENT

Property and equipment, net, consisted of the following:

	December 31,	
	2018	2017
	(in thousands)	
Lab equipment	$2,489	$2,229
Leasehold improvements	741	737
Computer hardware and software	428	355
Furniture and fixtures	159	77
Property and equipment, gross	3,817	3,398
Less: accumulated depreciation and amortization	(3,090)	(2,835)
Property and equipment, net	$727	$563

During the years ended December 31, 2018 and 2017, depreciation and amortization expense was $0.3 million. Depreciation and amortization expense is recorded primarily in research and development expense on the consolidated statements of operations and comprehensive loss.

6. ACCRUED LIABILITIES

Accrued liabilities consisted of the following:

	December 31,	
	2018	2017
	(in thousands)	
Accrued employee compensation and related taxes	$1,704	$1,538
Accrued outsourced clinical trials and preclinical studies	1,129	581
Accrued legal fees and expenses	376	185
Accrued other professional service fees	246	232
Deferred and accrued facility lease obligations	124	74
Value of liability-classified stock purchase warrants	82	82
Accrued equipment and lab materials	33	197
Other accrued liabilities	174	102
Total accrued liabilities	$3,868	$2,991

7. NOTES PAYABLE

2017 Silicon Valley Bank Loan Agreement

In November 2017, the Company entered into a loan and security agreement with Silicon Valley Bank (the “2017 SVB Loan Agreement”), which amended and restated the loan and security agreement Private Miragen entered into with Silicon Valley Bank in April 2015 (the “2015 SVB Loan Agreement”). Upon entry into the 2017 SVB Loan Agreement, the Company borrowed $10.0 million with a 30-month payment period following an 18-month interest-only payment

period ending in November 2021. Under specified circumstances, the interest-only period can be extended by an additional six months. Amounts

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outstanding bear interest at the prime rate (5.50% and 4.50% at December 31, 2018 and 2017, respectively), with a final payment fee equal to $0.9 million due upon maturity. As of December 31, 2018, no additional amounts are available under the 2017 SVB Loan Agreement.

The Company may elect to prepay prior to maturity all or any portion of the outstanding principal amounts under the 2017 SVB Loan Agreement, subject to a prepayment charge, depending on the date of prepayment or upon the occurrence of an event of default in which the Company's obligations to repay the outstanding principal is accelerated. The Company's obligations under the 2017 SVB Loan Agreement are secured by a first-priority security interest, right, and title in all business assets, excluding the Company's intellectual property, which is subject to a negative pledge.

The 2017 SVB Loan Agreement includes customary representations, warranties, and covenants (affirmative and negative), including restrictive covenants that limit the Company's ability to: encumber or dispose of the collateral securing the loan; change the business of the Company; transfer a material portion of the Company's assets; acquire other businesses; and merge or consolidate with or into any other business organization; incur additional indebtedness; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; enter into specified material transactions with Company affiliates; make non-ordinary course payments or enter into any amendment regarding subordinated debt of the Company; or become an "investment company" under the Investment Company Act of 1940, as amended; in each case subject to specified exceptions.

The 2017 SVB Loan Agreement also includes standard events of default, including payment defaults; breaches of covenants following any applicable cure period; material breaches of representations or warranties; the occurrence of a material adverse change (as defined in the 2017 SVB Loan Agreement); events relating to bankruptcy or insolvency; breaches of material third-party agreements; the occurrence of an unsatisfied material judgment against the Company; and specified governmental actions against the Company, including specified actions by the U.S. Food and Drug Administration. Upon the occurrence of an event of default, Silicon Valley Bank may declare all outstanding obligations immediately due and payable, including a prepayment charge, and take such other actions as are set forth in the 2017 SVB Loan Agreement. Upon the occurrence of an event of default, at the Silicon Valley Bank's discretion, interest on the 2017 SVB Loan Agreement will accrue at 5.0% above the rate that is otherwise applicable thereto until the earlier of the repayment of the Company's obligations under the 2017 SVB Loan Agreement or the cure of such event of default.

2015 Silicon Valley Bank Loan Agreement

In April 2015, Private Miragen entered into the 2015 SVB Loan Agreement and $5.0 million was funded in May 2015, which had a 30-month payment period following an 18-month interest-only payment period that ended in November 2016. Interest accrued on amounts outstanding at the prime rate minus 0.25%, with a final payment fee equal to 5.50% of amounts borrowed. Upon the execution of the 2017 SVB Loan Agreement, the 2015 SVB Loan agreement was terminated in its entirety. As a result, the Company paid the remaining principal and final interest payment with proceeds from the 2017 SVB Loan Agreement. The Company accounted for the termination of the 2015 SVB Loan Agreement as an extinguishment and incurred a loss on debt extinguishment of $0.1 million, which was recorded within interest expense.

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Amounts outstanding under the SVB loan agreements were as follows:

	December 31,	
	2018	2017
	(in thousands)	
Principal amount outstanding	$10,000	$10,000
Unamortized debt discount	(69)	(119)
Accreted final payment fee	367	41
Total note payable	10,298	9,922
Less: current maturities	(2,294)	—
Long-term note payable, net of current portion	$8,004	$9,922

Future annual minimum principal payments under the 2017 SVB Loan Agreement as of December 31, 2018 for the respective calendar years are as follows (in thousands):

2019	$2,333
2020	4,000
2021	3,667
Total	$10,000

8. COMMITMENTS AND CONTINGENCIES

Indemnification Agreements

The Company has entered into indemnification agreements with each of its directors and officers whereby it has agreed to indemnify such persons for certain events or occurrences while the individual is, or was, serving as a director, officer, employee, or other agent of the Company. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited.

Employment Agreements

The Company has entered into agreements with its executives and certain of its employees that provide for base salary, severance, eligibility for bonuses, and other generally available benefits. The agreements provide that the Company may terminate the employment of its employees, including executives, at any time, with or without cause.

If an employee under an employment agreement is terminated without cause, as defined in the employment agreements, or an employee under an employment agreement resigns for good reason, as defined in the employment agreements, then the employee under the employment agreement is entitled to receive, upon the execution of a release agreement, a severance package consisting of one or more of the following provisions: (i) the equivalent of up to 12 months of the employee’s base salary in effect immediately prior to date of termination; (ii) acceleration of vesting of the equivalent of up to 12 months of vesting of the executive’s outstanding unvested stock options or other equity awards that were outstanding as of the effective date of the executive’s employment agreement; and (iii) up to 12 months of continued health coverage.

If an executive is terminated without cause or resigns for good reason within one month prior to or 12 months following a change of control, as defined in the employment agreements, the executive is entitled to receive, upon the execution of a release agreement, a severance package consisting of: (i) the equivalent of 12 months of the executive’s base salary in effect immediately prior to date of termination; (ii) the vesting in full of the executive’s then-outstanding stock options or other equity awards subject to time-based vesting; and (iii) 12 months of continued health coverage. Solely in the case of the Company’s Chief Executive Officer, if such termination occurs one month before or 12 months following a change of control, then, upon the execution of a release agreement, the executive is entitled to:

(i) the equivalent of 24 months of the executive's base salary in effect immediately prior to the date of termination; (ii) the vesting in full of the executive's outstanding stock options or other equity awards subject to time-based vesting; and (iii) 12 months of continued health coverage.

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License Agreements with the University of Texas

As of December 31, 2018, the Company had two exclusive patent license agreements (the "UT License Agreements") with the Board of Regents of The University of Texas System (the "University of Texas"). Under each of the UT License Agreements, the University of Texas granted the Company exclusive and nonexclusive licenses to certain patent and technology rights. The University of Texas is a minority stockholder of the Company.

In consideration of rights granted by the University of Texas, the Company is required to: (i) pay a nonrefundable upfront license documentation fee in the amount of $10 thousand per license; (ii) pay an annual license maintenance fee in the amount of $10 thousand per license starting one year from the date of each agreement; (iii) reimburse the University of Texas for actual costs incurred in conjunction with the filing, prosecution, enforcement, and maintenance of patent rights prior to the effective date; and (iv) bear all future costs of and manage the filing, prosecution, enforcement, and maintenance of patent rights. During the year ended December 31, 2018 and 2017, the Company incurred immaterial upfront and maintenance fees, which were recorded as research and development expense. All costs related to the filing, prosecution, and maintenance of patent and technology rights are recorded as general and administrative expense when incurred.

Under the terms of the UT License Agreements, the Company may be obligated to make the following future milestone payments for each licensed product candidate: (i) up to approximately $0.6 million upon the initiation of defined clinical trials; (ii) $2.0 million upon regulatory approval in the United States; and (iii) $0.5 million per region upon regulatory approval in other specified regions. Additionally, if the Company or any of its sublicensees successfully commercializes any product candidate subject to the UT License Agreements, it is responsible for royalty payments in the low-single digits based upon net sales of such licensed products and payments at a percentage in the mid-teens of any sublicense income, subject to specified exceptions. The University of Texas's right to these royalty payments will expire as to each license agreement upon the expiration of the last patent claim subject to the applicable UT License Agreement. During the year ended December 31, 2018, the Company made an immaterial milestone payment, which is included in research and development expense in the Company's consolidated statements of operations and comprehensive loss. Prior to December 31, 2018, the Company did not incur any milestone payments.

The license term extends on a product-by-product and country-by-country basis until the expiration of the last to expire of the licensed patents that covers such product in such country. Upon expiration of the royalty payment obligation, the Company will have a fully-paid license in such country. The Company may also terminate each UT License Agreement for convenience upon a specified number of days' prior notice to the University of Texas. The University of Texas also has the right to earlier terminate the UT License Agreements after a defined date under specified circumstances where the Company has effectively abandoned its research and development efforts or has no sales. The UT License Agreements will terminate under customary termination provisions including automatic termination upon the Company's bankruptcy or insolvency, upon notice of an uncured material breach, and upon mutual written consent. All charges incurred under the UT License Agreements have been expensed to date due to the uncertainty as to future economic benefit from the acquired rights.

License Agreement with Roche Innovation Center Copenhagen A/S (formerly Santaris Pharma A/S)

In June 2010, Private Miragen entered into a license agreement with the Santaris Pharma A/S, which was subsequently acquired by F. Hoffmann-La Roche Ltd ("Roche") in 2014, and subsequently changed its name to Roche Innovation Center Copenhagen A/S ("RICC"). The agreement was amended in October 2011 and amended and restated in December 2012 (the "RICC License Agreement"). At the time the RICC License Agreement was entered into, Roche was a minority stockholder of the Company.

Under the RICC License Agreement, the Company has received exclusive and nonexclusive licenses from RICC to use specified technology of RICC (the "RICC Technology") for specified uses, including research, development, and commercialization of pharmaceutical products using this technology worldwide. Under the RICC License Agreement, the Company has the right to develop and commercialize the RICC Technology directed to four specified targets and the option to obtain exclusive product licenses for up to six additional targets. The acquisition of Santaris Pharma A/S by Roche was considered a change of control under the RICC License Agreement, and as such, certain terms and conditions of the RICC License Agreement changed, as contemplated and in accordance with the RICC License Agreement. These changes primarily relate to milestone payments reflected in the disclosures below. If the Company exercises its option to obtain additional product licenses or to replace the target families, it will be required to make additional payments to RICC.

Under the terms of the RICC License Agreement, milestone payments were previously decreased by a specified percentage as a result of the change of control by RICC referenced above. The Company is obligated to make milestone payments for each licensed product for up to $5.2 million, which is inclusive of a potential product license option fee. Certain of these milestones

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will be increased by a specified percentage if the Company undergoes a change of control during the term of the RICC License Agreement. If the Company grants a third party a sublicense to the RICC Technology, it is required to remit to Roche up to a specified percentage of the upfront and milestone and other specified payments it receives under its sublicense, and if such sublicense covers use of the RICC Technology in the United States or the entire European Union, the Company will not have any further obligation to pay the fixed milestone payments noted above. During the year ended December 31, 2018, the Company incurred $0.7 million in expense related to a milestone reached, which is included in research and development expense in the Company's consolidated statements of operations and comprehensive loss.

If the Company or its sublicensee successfully commercializes any product candidate subject to the RICC License Agreements, then RICC is entitled to royalty payments in the mid-single digits on the net sales of such product, provided that if such net sales are made by a sublicensee under the RICC License Agreement, RICC is entitled to royalty payments equal to the lesser of a percentage in the mid-single digits on the net sales of such product or a specified percentage of the royalties paid to the Company by such sublicensee, subject to specified restrictions. The Company is obligated to make any such royalty payments until the later of: (i) a specified anniversary of the first commercial sale of the applicable product or (ii) the expiration of the last valid patent claim licensed by RICC under the RICC License Agreement underlying such product. Upon the occurrence of specified events, the royalty owed to RICC will be decreased by a specified percentage.

The RICC License Agreement will terminate upon the latest of the expiration of all of RICC's royalty rights, the termination of the last Miragen target, or the expiration of its right to obtain a product license for a new target under the RICC License Agreement. The Company may also terminate the RICC License Agreement for convenience upon a specified number of days' prior notice to RICC, subject to specified terms and conditions. Either party may terminate the RICC License Agreement upon an uncured material breach by the other party and RICC may terminate the RICC License Agreement upon the occurrence of other specified events immediately or after such event is not cured within a specified number of days, as applicable.

All charges incurred under the RICC License Agreement have been expensed to date due to the uncertainty as to future economic benefit from the acquired rights.

During the years ended December 31, 2018 and 2017, the Company paid $0.3 million and $0.6 million, respectively, to RICC for raw materials to be used in its drug manufacturing process.

Subcontract Agreement with Yale University

In October 2014, Private Miragen and Yale University ("Yale") entered into a subcontract agreement and then into a subaward agreement in March 2015 (the "Yale Agreements"), which were subsequently amended. Under the Yale Agreements, the Company is providing specified services regarding the development of a proprietary compound that targets microRNA-29 in the indication of idiopathic pulmonary fibrosis. Yale entered into the Yale Agreements in connection with a grant that Yale received from the National Institutes of Health ("NIH") for the development of a microRNA-29 mimic as a potential therapy for pulmonary fibrosis.

In consideration of the Company's services under the Yale Agreements, Yale has agreed to reimburse the Company up to a specified amount over five years, subject to the availably of funds under the grant and continued eligibility. Under the terms of the Yale Agreements, the Company retains all rights to any and all intellectual property developed solely by the Company in connection with the Yale Agreements. Yale has also agreed to provide the Company with an exclusive option to negotiate in good faith for an exclusive, royalty-bearing license from Yale for any intellectual property developed by Yale or jointly by the parties under the Yale Agreements. Yale is responsible for filing, prosecuting, and maintaining foreign and domestic patent applications and patents on all inventions jointly developed

by the parties under the Yale Agreements. Through December 31, 2018, the Company received $0.9 million under the Yale Agreements.

The Yale Agreements terminate automatically on the date that Yale delivers its final research report to the NIH under the terms of the grant underlying the Yale Agreements. Each party may also terminate the Yale Agreements upon a specified number of days' notice in the event that the NIH's grant funding is reduced or terminated or upon material breach by the other party.

License Agreements with the t2cure GmbH

In October 2010, Private Miragen entered into a license and collaboration agreement (the "t2cure Agreement") with t2cure GmbH ("t2cure"), which was subsequently amended. Under the t2cure Agreement, the Company received a worldwide, royalty-bearing, and exclusive license to specified patent and technology rights relating to microRNA-92.

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In consideration of rights granted by t2cure, Private Miragen paid an upfront fee of $46 thousand and agreed to: (i) pay an annual license maintenance fee in the amount of €3 thousand ($3 thousand as of December 31, 2018); and (ii) reimburse t2cure for costs incurred in conjunction with the filing, prosecution, enforcement, and maintenance of patent rights.

Under the terms of the t2cure Agreement, the Company is obligated to make the following future milestone payments for each licensed product, as defined in the t2cure Agreement: (i) up to approximately $0.7 million upon the initiation of certain defined clinical trials; (ii) $2.5 million upon regulatory approval in the United States; and (iii) up to $1.5 million per region upon regulatory approval in the European Union or Japan. Additionally, if the Company or any of its sublicensees successfully commercialize any product candidate subject to the t2cure Agreement, it is responsible for royalty payments equal to percentages in the low-single digits upon net sales of licensed products, and under specified circumstances, sublicense fees equal to a percentage in the low twenties of sublicense income received by it. The Company is obligated to make any such royalty payment until the later of: (i) the tenth anniversary of the first commercial sale of the applicable product or (ii) the expiration of the last valid claim to a patent licensed by t2cure under the t2cure Agreement covering such product. If such patent claims expire prior to the end of the ten-year term, then the royalty owed to t2cure will be decreased by a specified percentage. The Company also has the right to decrease its royalty payments by a specified percentage for royalties paid to third parties for licenses to certain third-party intellectual property.

The license term extends on a country-by-country basis until the later of: (i) the tenth anniversary of the first commercial sale of a licensed product in a country and (ii) the expiration of the last to expire valid claim that claims such licensed product in such country. Upon expiration of the royalty payment obligation, the Company will have a fully-paid license in such country. The Company has the right to terminate the t2cure Agreement at will, on a country-by-country basis, after 60 days' written notice. The t2cure Agreement will also automatically terminate upon the Company's bankruptcy or insolvency or upon notice of an uncured material breach.

All charges incurred under the t2cure Agreement have been expensed to date, due to the uncertainty as to future economic benefit from the acquired rights.

License Agreement with The Brigham and Women's Hospital

In May 2016, Private Miragen and The Brigham and Women's Hospital ("BWH") entered into an exclusive patent license agreement (the "BWH License Agreement"). Under the BWH License Agreement, the Company has an exclusive, worldwide license, including a right to sublicense, to specified patent rights and a nonexclusive, worldwide license, including a right to sublicense, to specified technology rights of BWH, each related to certain microRNAs believed to be involved in various neurodegenerative disorders. As consideration for these rights, the Company is obligated to pay a specified annual license fee. BWH is also entitled to milestone payments of up to approximately $2.6 million for each of the Company's product candidates developed based on the patent rights subject to the BWH License Agreement plus a one-time sales milestone payment of $0.3 million for all product candidates developed based on the patent rights subject to the BWH License Agreement. If the Company were to successfully commercialize any product candidate subject to the BWH License Agreement, then BWH is entitled to royalty payments in the low-single digits on the net sales of such product. BWH's right to these royalty payments will expire on a product-by-product and country-by-country basis upon the expiration of the last patent claim in such country that is subject to the BWH License Agreement and covers the product, and the Company's license to such product in such country will become fully paid at such time. BWH is also entitled to a percentage in the low-double digits of any sublicense income from such product, subject to specified exceptions. The Company is also responsible for all costs associated with the preparation, filing, prosecution, and maintenance of the patent rights subject to the BWH License Agreement. Additionally, the Company is obligated to use commercially-reasonable efforts to develop a product under the BWH License Agreement and to meet specified diligence milestones thereunder.

The BWH License Agreement will terminate upon the expiration of all issued patents and patent applications subject to the patent rights under the agreement. The Company may also terminate the BWH License Agreement for convenience upon a specified number of days' prior notice to BWH. BWH may terminate the BWH License Agreement upon a breach by the Company of its payment obligations and upon the occurrence of other specified events that are not cured within a specified number of days, provided that such termination is automatic upon the Company's bankruptcy or insolvency.

During the years ended December 31, 2018 and 2017, the Company paid annual license fees, which were immaterial.

Facility Lease

In December 2010, Private Miragen entered into a multi-year, noncancelable lease agreement for its current office and lab space. The agreement was subsequently amended to extend the term through August 2020. The lease agreement includes rent

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escalation clauses through the lease term and an option to extend the lease term for up to two terms of three years each. Minimum base lease payments, including the impact of tenant improvement allowances, under the operating lease are recognized on a straight-line basis over the full term of the lease.

During the years ended December 31, 2018 and 2017, rent expense was $0.3 million. The Company is also required to pay for operating expenses related to the leased space, which were $0.3 million for the year ended December 31, 2018 and 2017.

Future annual minimum payments under the lease as of December 31, 2018 for the respective calendar year were as follows (in thousands):

2019	$404
2020	277
Total	$681

9. CAPITAL STOCK

Common Stock

The Company is authorized to issue 105,000,000 shares of its stock, of which 100,000,000 shares have been designated as Common Stock and 5,000,000 shares have been designated as preferred stock with a par value of $0.01 per share. The number of authorized shares of Common Stock may be increased or decreased by the affirmative vote of the holders of a majority of the Company's stock who are entitled to vote. Each share of Common Stock is entitled to one vote. The holders of Common Stock are entitled to receive dividends when and as declared or paid by its board of directors.

Common Stock Purchase Agreement

In August 2018, the Company and The Leukemia & Lymphoma Society, Inc. ("LLS") entered into a Common Stock Purchase Agreement (the "LLS Stock Purchase Agreement") for the sale of up to $5.0 million of shares of Common Stock to LLS in a private placement (the "Offering"). Under the terms of the LLS Stock Purchase Agreement, the Company expects to raise up to approximately $5.0 million in gross proceeds by selling shares of Common Stock to LLS in up to five separate closings. The initial closing of the Offering was held on August 6, 2018. At the initial closing, the Company issued 150,987 shares of Common Stock at a price per share equal to $6.62, which resulted in net proceeds of approximately $0.9 million after expenses incurred in connection with the Offering. The price per share of Common Stock to be sold in any subsequent closing will be equal to the average of the volume weighted-average prices of a share of Common Stock on the Nasdaq Capital Market for the three trading days beginning with the first trading day after the date of achievement of the relevant milestone for each such closing. Each closing is subject to the Company's achievement of specified operational milestones under the LLS Stock Purchase Agreement and other customary closing conditions, provided, however, that each such closing must be completed prior to December 31, 2021.

Common Stock Sales Agreement

In March 2017, the Company entered into an at the market issuance Common Stock Sales Agreement (the "ATM Agreement") with Cowen and Company, LLC ("Cowen") under which the Company may offer and sell, from time to time at its sole discretion, shares of its Common Stock having an aggregate offering price of up to $50.0 million through Cowen as its sales agent.

Cowen may sell the Common Stock by any method permitted by law deemed to be an “at the market offering” as defined in Rule 415 of the Securities Act of 1933, as amended, including without limitation sales made by means of ordinary brokers’ transactions on The Nasdaq Capital Market or otherwise at market prices prevailing at the time of sale, in block transactions, or as otherwise directed by the Company. Cowen will use commercially-reasonable efforts to sell the Common Stock from time to time, based upon instructions from the Company (including any price, time, or size limits or other customary parameters or conditions the Company may impose). The Company will pay Cowen a commission equal to 3.0% of the gross sales proceeds of any Common Stock sold through Cowen under the ATM Agreement. The Company also has provided Cowen with customary indemnification rights.

The Company is not obligated to make any sales of Common Stock under the ATM Agreement. The offering of shares of Common Stock pursuant to the ATM Agreement will terminate upon the earlier of: (i) the sale of all Common Stock subject to the ATM Agreement or (ii) termination of the ATM Agreement in accordance with its terms.

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During the year ended December 31, 2018, the Company sold, pursuant to the terms of the ATM Agreement, 372,852 shares of Common Stock, at a weighted average price of $7.37 per share, for aggregate net proceeds of approximately $2.7 million, including commissions to Cowen as sales agent. Since March 2017 and through December 31, 2018, the Company sold, pursuant to the terms of the ATM Agreement, an aggregate of 1,213,386 shares of Common Stock, at a weighted average price of $8.74 per share, for aggregate net proceeds of approximately $10.2 million, including initial expenses for executing the “at the market offering” and commissions to Cowen as sales agent.

Common Stock Public Offering

In February 2018, the Company entered into an underwriting agreement relating to a public offering of its Common Stock, pursuant to which the Company sold 7,414,996 shares of Common Stock at a price of $5.50 per share, which resulted in net proceeds of approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses payable by the Company.

Private Miragen Common Stock Offering

In February 2017, immediately prior to the Merger and in accordance with subscription agreements entered into with certain investors in October 2016, Private Miragen issued and sold an aggregate of 9,045,126 shares of Private Miragen’s common stock at a price per share of $4.50, or 6,359,628 shares of Common Stock at a price per share of $6.40 as adjusted for the exchange ratio in the Merger, which resulted in net proceeds of approximately $39.2 million, after giving effect to associated financing fees of $1.5 million.

Series Preferred

In February 2017, in conjunction with the Merger, all of the outstanding redeemable convertible preferred stock of Private Miragen converted into Private Miragen common stock at a ratio of 1:1 and was immediately exchanged for Common Stock at an exchange ratio of 0.7031 as a result of the Merger. A summary of the conversion by class of preferred stock is summarized as follows (in thousands, except share data):

	Series A		Series B		Series C		Total	
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount
Balance at December 31, 2016	7,149,176	$23,124	2,166,651	$12,975	9,268,563	$40,877	18,584,390	$76,976
Accretion of redeemable convertible preferred stock to redemption value	—	1	—	1	—	3	—	5
Conversion of preferred stock to common stock	(7,149,176)	(23,125)	(2,166,651)	(12,976)	(9,268,563)	(40,880)	(18,584,390)	(76,981)
Balance at February 13, 2017	—	$—	—	$—	—	$—	—	$—

As of December 31, 2018, the Company had no shares of preferred stock outstanding and had not designated any class or series of preferred stock. Under the Company’s amended and restated certificate of incorporation, the Company’s board of directors has the authority to designate and issue up to 5,000,000 shares of preferred stock, at its discretion, in one or more classes or series and to fix the powers, preferences and rights, and the qualifications, limitations, or restrictions thereof, including dividend rights, conversion rights, voting rights, terms of redemption, and liquidation preferences, without further vote or action by the Company’s stockholders.

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10. WARRANTS

Stock purchase warrant activity is as follows:

	Common Stock Warrants		Preferred Stock Warrants	
	Number	Weighted Average Exercise Price	Number	Weighted Average Exercise Price
Outstanding at December 31, 2016	7,031	$ 0.57	25,779	$ 6.21
Warrants acquired in Merger	13,534	$ 80.70	—	$ —
Preferred stock warrants converted into Common Stock warrants	25,779	$ 6.21	(25,779)	$ 6.21
Granted	24,097	$ 7.15	—	$ —
Exercised	(21,092)	$ 3.04	—	$ —
Outstanding at December 31, 2017	49,349	$ 27.65	—	$ —
Activity	—	$ —	—	$ —
Outstanding at December 31, 2018	49,349	$ 27.65	—	$ —

A summary of outstanding Common Stock purchase warrants as of December 31, 2018 is as follows:

Number of Underlying Shares	Exercise Price	Expiration Date
13,534	$80.70	2019 & 2020
11,718	$8.53	2025
24,097	$7.15	2024
49,349		

11. SHARE-BASED COMPENSATION

Equity Incentive Plans

As of December 31, 2018, there were 1,639,796 options outstanding and no remaining equity awards available for future issuances under the 2008 Plan. All awards granted under the 2008 Plan that, after February 13, 2017, expire or terminate for any reason prior to exercise or settlement, are forfeited, or are reacquired, withheld, or not issued to satisfy a tax withholding obligation or to satisfy the exercise price of a stock award, will become available for grant under the 2016 Plan in accordance with its terms.

The 2016 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, other stock awards, and performance awards that may be settled in cash, stock, or other property. All employees and non-employee directors are eligible to participate in the 2016 Plan and may receive all types of awards other than incentive stock options. Incentive stock options may be granted under the 2016 Plan only to employees (including officers) and employees of the Company’s affiliates.

The aggregate number of shares of Common Stock that may be issued under the 2016 Plan will not exceed 4,182,404 shares, which number is the sum of: (i) 1,681,294 shares, plus (ii) the number of shares subject to outstanding stock awards that were granted under the 2008 Plan, that, from and after the closing date of the Merger, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to meet a contingency or condition required to vest such shares, or are reacquired, withheld, or not issued to satisfy a tax withholding obligation in connection with an award or to satisfy the purchase price or exercise price of a stock award, if any, as such shares

become available from time to time, plus (iii) shares from automatic increases to the share reserve, as described in more detail below. In accordance with the 2016 Plan, the share reserve will automatically increase on January 1 of each year, for a period of not more than ten years, commencing on January 1 of the year following the year in which the effective date of the 2016 Plan occurs, and ending on (and including) January 1, 2026, in an amount equal to 4% of the shares of Common Stock outstanding on December 31 of the preceding calendar year; however, the board of directors or compensation committee may act prior to January 1 of a given year to provide that there will be no January 1 increase in the share reserve for such year or that the increase in the share reserve for such year will be a lesser number of shares of Common Stock than would otherwise occur pursuant to the automatic increase. On January 1, 2018 and 2019, the share reserve had automatic increases of 902,720 and 1,233,578 shares, respectively. As of December 31, 2018, there

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were outstanding stock options to purchase 1,887,617 shares of Common Stock and 705,317 shares of Common Stock available for issuance pursuant to the terms under the 2016 Plan.

Options granted under the 2008 Plan and 2016 Plan have an exercise price equal to the market value of the Common Stock at the date of grant and expire ten years from the date of grant. Generally, options vest 25% on the first anniversary of the vesting commencement date and 75% ratably in equal monthly installments over the remaining 36 months. The Company has also granted options that vest in equal monthly or quarterly amounts over periods up to 48 months.

A summary of Common Stock option activity is as follows:

	Number of Options (in thousands)	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2016	2,321	$ 1.44		
Granted	989	$ 11.37		
Exercised	(412)	$ 0.74		
Forfeited	(35)	$ 11.36		
Outstanding at December 31, 2017	2,863	$ 4.85		
Granted	1,108	$ 7.37		
Exercised	(280)	$ 0.65		
Forfeited or canceled	(164)	$ 9.52		
Outstanding at December 31, 2018	3,527	$ 5.76	6.95	$ 2,698
Vested or expected to vest at December 31, 2018	3,527	$ 5.76	6.95	$ 2,698
Exercisable as of December 31, 2018	1,979	$ 4.20	5.61	$ 2,421

The total intrinsic value of stock options exercised during the year ended December 31, 2018 and 2017 was $1.6 million and $3.8 million, respectively. Cash received from the exercise of stock options during the year ended December 31, 2018 and 2017 was approximately $0.2 million and $0.3 million respectively.

Fair Value Assumptions

The Company uses the Black-Scholes option pricing model to estimate the fair value of stock options granted under its equity compensation plans. The Black-Scholes model requires inputs for risk-free interest rate, dividend yield, volatility, and expected lives of the options. Because the Company has a limited history of stock purchase and sale activity, expected volatility is based on historical data from public companies that are similar to the Company in size and nature of operations. The Company will continue to use similar entity volatility information until its historical volatility is relevant to measure expected volatility for option grants. The Company accounts for forfeitures as they occur. The risk-free rate for periods within the contractual life of each option is based on the U.S. Treasury yield curve in effect at the time of the grant for a period commensurate with the expected term of the grant. The expected term (without regard to forfeitures) for options granted represents the period of time that options granted are expected to be outstanding and is derived from the contractual terms of the options granted and expected option-exercise behaviors. Prior to the Merger, Private Miragen estimated the fair value of underlying shares of its common stock using a third-party valuation report that derived the fair value using the probability-weighted expected return method. After the Merger, the fair value of the underlying Common Stock is based on the closing price of the Common Stock on The Nasdaq Capital Market at the date of grant.

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Stock Options Granted to Employees and Directors

The weighted-average grant-date fair value of options granted to the Company's employees and members of its board of directors during the years ended December 31, 2018 and 2017 was $5.41 and $8.27, respectively. The fair value was determined by the Black-Scholes option pricing model using the following weighted-average assumptions:

	Year Ended December 31,	
	2018	2017
Expected term, in years	6.35	6.43
Expected volatility	85.2 %	83.8 %
Risk-free interest rate	2.6 %	2.1 %
Expected dividend yield	— %	— %
Weighted-average exercise price	$7.37	$11.37

Stock Options Granted to Non-Employees

The Company determines the value of Common Stock options issued to non-employees (other than members of its board of directors) using the Black-Scholes option pricing model and adjusting the value of such awards to current fair value each reporting period until the awards are vested or a performance commitment has otherwise been reached. No Common Stock options were issued to non-employees during the years ended December 31, 2018 and 2017.

Employee Stock Purchase Plan

The 2016 Employee Stock Purchase Plan ("ESPP") allows qualified employees to purchase shares of the Company's Common Stock at a price equal to 85% of the lower of: (i) the closing price at the beginning of the offering period or (ii) the closing price at the end of the offering period. The Company expects that a new 6-month offering period will begin each August 22 and February 22. As of December 31, 2018, the Company had 0.4 million shares available for issuance and 66 thousand shares had been issued under the ESPP.

Share-Based Compensation Expense

Share-based compensation related to all equity awards issued pursuant to the 2008 Plan and 2016 Plan and for estimated shares to be issued under the ESPP for the purchase periods active during each respective period is included in the consolidated statements of operations and comprehensive loss as follows:

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Research and development	$1,286	$917
General and administrative	2,393	1,492
Total share-based compensation expense	$3,679	$2,409

As of December 31, 2018, the Company had $8.3 million of total unrecognized employee and non-employee share-based compensation costs, which the Company expects to recognize over a weighted-average remaining period of 2.6 years.

12. NET LOSS PER SHARE

Basic net loss per share is computed by dividing the net loss available to common stockholders by the weighted-average number of Common Stock outstanding. Diluted net loss per share is computed similarly to basic net loss per share except that the denominator is increased to include the number of additional shares of Common Stock that would have been outstanding if the potential shares of Common Stock had been issued and if the additional shares of Common Stock were dilutive. Diluted net loss per share is the same as basic net loss per share of Common Stock, as the effects of potentially dilutive securities are antidilutive.

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Potentially dilutive securities include the following:

	December 31, 2018	2017
	(in thousands)	
Options to purchase Common Stock	3,527	2,863
Warrants to purchase Common Stock	49	49
Total	3,576	2,912

13. INCOME TAXES

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to reverse. As a result of the reduction in the federal tax rate, the Company remeasured its ending net deferred tax assets and liabilities as of December 31, 2017. This remeasurement resulted in a $10.8 million revaluation to net deferred tax assets, which was equally offset by a reduction to the recorded valuation allowance.

Since its inception, the Company has incurred net taxable losses, and accordingly, no current provision for income taxes has been recorded. This amount differs from the amount computed by applying the U.S. federal income tax rate of 21.0% to pretax loss due to the provision of a valuation allowance to the extent of the Company's net deferred tax asset, as well as to state income taxes and nondeductible expenses.

The effective income tax rate of the provision for income taxes differs from the federal statutory rate as follows:

	Year Ended December 31,	
	2018	2017
Federal statutory income tax rate	21.0 %	35.0 %
Federal and state tax credits	10.3	(4.8)
State income taxes, net of federal benefit	3.0	3.1
Change in valuation allowance	(32.5)	14.5
Other permanent items	(1.0)	1.9
Change in tax rate	(0.7)	(40.7)
Transaction costs	—	(2.9)
Net operating loss reduction	—	(6.7)
Other, net	(0.1)	0.6
Effective income tax rate	— %	— %

The tax effects of temporary differences that give rise to significant portions of the deferred income tax assets and liabilities are presented below:

	Year Ended December 31,	
	2018	2017
	(in thousands)	
Net operating loss carryforwards	$24,137	$18,257
Tax credits	6,571	2,287
Accruals and reserves	854	697
Stock-based expense	876	439
Start-up costs	698	829

Gross deferred tax assets	33,136	22,509
Valuation allowance	(33,136)	(22,509)
Net deferred tax assets	$—	$—

At December 31, 2018, the Company had approximately $97.9 million and $6.6 million of net operating loss and research and experimentation tax carryforwards, respectively, which will begin to expire in 2028. In addition, the realization of net operating

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losses to offset potential future taxable income and related income taxes that would otherwise be due is subject to annual limitations under the provisions of Internal Revenue Code Sections 382 and 383 and similar state provisions, which may result in the expiration of additional net operating losses before future utilization as a result of ownership changes. As a result of these ownership change provisions, the Company estimated an aggregate limitation on the utilization of net operating losses of $4.6 million. In addition to the limitation of net operating losses of $4.6 million, approximately $2.8 million of research and development tax credits were derecognized with the inability of the Company to ever realize a benefit from those credits in the future.

As of December 31, 2018 and 2017, the Company's net deferred tax assets before valuation allowance was $33.1 million and $22.5 million, respectively. In assessing the realizability of its deferred tax assets, the Company considers whether it is more likely than not that some portion or all of its deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences become deductible. The Company considers the scheduled reversal of deferred tax liabilities, projected future taxable income, and tax planning strategies in making this assessment. As the Company does not have any historical taxable income or projections of future taxable income over the periods in which the deferred tax assets are deductible, and after consideration of its history of operating losses, the Company does not believe it is more likely than not that it will realize the benefits of its net deferred tax assets, and accordingly, has established a valuation allowance equal to 100% of its net deferred tax assets at December 31, 2018 and 2017. The change in valuation allowance was an increase of $10.6 million in 2018 and a decrease of $3.8 million in 2017.

The Company has concluded that there were no significant uncertain tax positions relevant to the jurisdictions where it is required to file income tax returns requiring recognition in the consolidated financial statements for the years ended 2018 and 2017. As of December 31, 2018 and 2017, the Company had no accrued interest related to uncertain tax positions.

The Company's federal and state returns for 2014 through 2018 remain open to examination by tax authorities.

14. SELECTED QUARTERLY FINANCIAL DATA (UNAUDITED)

The tables below summarize the Company's unaudited quarterly operating results for the year ended December 31, 2018:

	For the Quarters Ended			
	March	June	September	December
	(in thousands)			
Revenue	$4,784	$2,182	$ 944	$476
Research and development expenses	(6,413)	(8,375)	(7,399)	(8,234)
General and administrative expenses	(2,990)	(2,668)	(2,696)	(2,695)
Other income (expense), net	(42)	147	140	136
Net loss	$(4,661)	$(8,714)	$ (9,011)	$(10,317)
Net loss available to common stockholders	$(4,661)	$(8,714)	$ (9,011)	$(10,317)
Net loss per share, basic and diluted	$(0.18)	$(0.29)	$ (0.29)	$(0.33)

The tables below summarize the Company's unaudited quarterly operating results for the year ended December 31, 2017:

	For the Quarters Ended			
	March	June	September	December
	(in thousands)			
Revenue	$462	$718	$ 1,631	$ 1,192
Research and development expenses	(4,120)	(5,487)	(5,018)	(4,998)

General and administrative expenses	(3,281)	(2,581)	(2,502)	(2,548)
Other income (expense), net	$(41)	$38	$55	$(32)
Net loss	$(6,980)	$(7,312)	$(5,834)	$(6,386)
Net loss available to common stockholders	$(6,985)	$(7,312)	$(5,834)	$(6,386)
Net loss per share, basic and diluted	$(0.60)	$(0.34)	$(0.27)	$(0.29)

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15. SUBSEQUENT EVENTS

The Company has evaluated subsequent events and has determined there are no other subsequent events requiring disclosure.