Dicerna Pharmaceuticals Inc
Form 10-K
March 13, 2019
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

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
TRANSITION REPORTS PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to
Commission File Number: 001-36281

DICERNA PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	20-5993609
(State or other jurisdiction of incorporation or organization)	(IRS Employer Identification No.)

87 Cambridgepark Drive, Cambridge, MA 02140
(Address of principal executive offices and zip code)
(617) 621-8097
(Registrant's telephone number, including area code)

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

Title of Each Class	Name of Each Exchange on Which Registered
Common Stock, $0.0001 par value	The Nasdaq Global Select Market

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

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

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. x

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

Large accelerated filer	Accelerated filer	x
Non-accelerated filer	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. x

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

The aggregate market value of the registrant's common stock held by non-affiliates of the registrant on June 30, 2018 was approximately $442.8 million based on the last reported sale of the registrant's common stock on The Nasdaq Global Select Market on June 30, 2018 of $12.25 per share.

As of March 4, 2019, there were 68,264,949 shares of the registrant's common stock, par value $0.0001 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for its 2019 Annual Meeting of Stockholders are incorporated by reference into Part III hereof. Such proxy statement will be filed with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K includes "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements other than statements of historical fact are "forward-looking statements" for purposes of this Annual Report on Form 10-K. In some cases, you can identify forward-looking statements by terminology such as "may," "could," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "intend," "contemplate," "project," "continue," "potential," "ongoing," "goal," or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- how long we expect to maintain liquidity to fund our planned level of operations and our ability to obtain additional funds for our operations;
- the initiation, timing, progress and results of our research and development programs, preclinical studies, any clinical trials and Investigational New Drug application ("IND"), Clinical Trial Application ("CTA"), New Drug Application ("NDA") and other regulatory submissions;
- our ability to identify and develop product candidates for treatment of additional disease indications;
- our or a collaborator's ability to obtain and maintain regulatory approval of any of our product candidates;
- the rate and degree of market acceptance of any approved product candidates;
- the commercialization of any approved product candidates;
- our ability to establish and maintain additional collaborations and retain commercial rights for our product candidates in the collaborations;
- the implementation of our business model and strategic plans for our business, technologies and product candidates;
- our estimates of our expenses, ongoing losses, future revenue and capital requirements;
- our ability to obtain and maintain intellectual property protection for our technologies and product candidates and our ability to operate our business without infringing the intellectual property rights of others;
- our reliance on third parties to conduct our preclinical studies or any clinical trials;
- our reliance on third-party suppliers and manufacturers to supply the materials and components for, manufacture, and research and, develop our preclinical and clinical trial drug supplies;
- our ability to attract and retain qualified key management and technical personnel;
- our dependence on our existing collaborators, Eli Lilly and Company ("Lilly"), Alexion Pharmaceuticals, Inc. (together with its affiliates, "Alexion"), and Boehringer Ingelheim International GmbH ("BI"), for developing, obtaining regulatory approval for and commercializing product candidates in the collaborations;
- our receipt and timing of any potential milestone payments or royalties under our existing research collaborations and license agreements or any future arrangements with our existing collaboration partners or any other collaborators;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act;
- our financial performance; and
- developments relating to our competitors or our industry.

These statements relate to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those set forth in Part I, Item 1A – "Risk Factors" below and for the reasons described elsewhere in this Annual Report on Form 10-K. Any forward-looking statement in this Annual Report on Form 10-K reflects our current view with respect to future events and is subject to these and other risks, uncertainties and assumptions relating to our operations, results of operations, industry and future growth. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Annual Report on Form 10-K also contains estimates, projections and other information concerning our industry, our business and the markets for certain drugs, including data regarding the estimated size of those markets, their projected growth rates and the incidence of certain medical conditions. Information that is based on estimates,

forecasts, projections or similar methodologies

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is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained these industry, business, market and other data from reports, research surveys, studies and similar data prepared by third parties, industry, medical and general publications, government data and similar sources. In some cases, we do not expressly refer to the sources from which these data are derived.

Except where the context otherwise requires, in this Annual Report on Form 10-K, “we,” “us,” “our,” “Dicerna,” and the “Company” refer to Dicerna Pharmaceuticals, Inc. and, where appropriate, its consolidated subsidiaries.

Trademarks

This Annual Report on Form 10-K includes trademarks, service marks, and trade names owned by us or other companies. All trademarks, service marks, and trade names included in this Annual Report on Form 10-K are the property of their respective owners.

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PART I

ITEM 1. BUSINESS

Overview

Dicerna™ Pharmaceuticals, Inc. ("we," "us," "our," the "Company," or "Dicerna") is a biopharmaceutical company focused on discovery and development of innovative, subcutaneously delivered ribonucleic acid ("RNA") interference ("RNAi")-based therapeutics using our GalXC™ RNAi platform for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases. Within these therapeutic areas, we believe our GalXC RNAi platform will allow us to build a broad pipeline of therapeutics designed to have attractive pharmaceutical properties, including a subcutaneous route of administration, infrequent dosing (e.g., dosing that is monthly or quarterly, and potentially even less frequent), high therapeutic index, and target a single gene. Our key development programs include DCR PHXC for the treatment of primary hyperoxaluria ("PH"), currently in a Phase 1 clinical trial with expected initiation of registration studies in the first quarter of 2019; DCR HBVS for the treatment of chronic hepatitis B virus ("HBV"), currently in a Phase 1 clinical trial; and an undisclosed product candidate against a serious rare liver disease, currently in Clinical Trial Application ("CTA") or Investigational New Drug application ("IND") enabling studies. Dicerna intends to discover, develop, and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. Dicerna has strategic collaborations with Eli Lilly and Company ("Lilly"), Alexion Pharmaceuticals, Inc. (together with its affiliates, "Alexion"), and Boehringer Ingelheim International GmbH ("BI").

All of our GalXC drug discovery and development efforts are based on the therapeutic modality of RNAi, a highly potent and specific mechanism for silencing the activity of a targeted gene. In this naturally occurring biological process, double-stranded RNA molecules induce the enzymatic destruction of the messenger ribonucleic acid ("mRNA") of a target gene that contains sequences that are complementary to one strand of the therapeutic double-stranded RNA molecule. Our approach is to design proprietary double-stranded RNA molecules that have the potential to engage the enzyme Dicer and initiate an RNAi process to silence a specific target gene. Our GalXC RNAi platform utilizes a proprietary structure of double-stranded RNA molecules configured for subcutaneous delivery to the liver. Due to the enzymatic nature of RNAi, a single GalXC molecule incorporated into the RNAi machinery can destroy hundreds or thousands of mRNAs from the targeted gene.

Strategy

We are committed to delivering transformative therapies based on our GalXC RNAi platform to patients with diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases. We have qualified dozens of disease-associated genes with clinical indications, in which we believe an RNAi-based inhibitor may provide substantial benefit to patients by providing additional therapeutic opportunities. The key elements of our strategy are as follows:

- Create new programs in indication areas with high unmet medical need. We intend to continue to use our proprietary GalXC RNAi technology platform to create new, high value pharmaceutical programs. Our areas of primary focus are: (1) rare inherited diseases involving genes in the liver; (2) other therapeutic areas involving the expression of therapeutic gene targets in the liver such as viral infectious diseases, chronic liver diseases, and cardiovascular diseases; and (3) further leveraging our successes with the GalXC platform to explore non-hepatic therapeutic gene targets.
- Validate our product candidates and our platform in clinical proof-of-concept studies. On September 5, 2018, we declared attainment of clinical proof-of-concept for DCR PHXC (which is in development for all forms of PH) and intend to demonstrate proof-of-concept for our other development programs starting in 2019. Based on precedents in the RNAi field, we are optimistic that our preclinical studies, which showed the significant knockdown of target mRNA activity lasting for up to three months after the last dose and disease biomarker activity, potentially may translate into beneficial clinical results for multiple programs.
- Retain significant portions of the commercial rights for certain rare disease programs. We seek to retain, subject to the evaluation of potential licensing opportunities as they may arise, a full or substantial ownership stake and to invest internally in diseases with focused patient populations, such as certain rare diseases. These certain rare diseases represent opportunities that carry a relatively higher probability of success, with genetically and molecularly defined

disease markets, high unmet need, a limited number of centers of excellence to facilitate reaching these patients, and the potential for more rapid clinical development programs.

- Enter into collaborations with pharmaceutical companies either for our GalXC RNAi technology platform or specific indications or therapeutic areas. For more complex diseases with multiple gene dysfunctions and larger patient populations, we plan to pursue collaborations that can provide the enhanced scale, resources, and commercial infrastructure required to maximize these prospects, such as our existing collaborative research and license agreements.

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We may establish collaborations with pharmaceutical companies across multiple programs or specific indication areas, either before or after clinical proof-of-concept, depending on the attractiveness of the opportunities. These collaborations have the potential to provide us with further validation of our technology platform, funding to advance our proprietary product candidates, or access to development, manufacturing, and commercial capabilities.

- Expand the reach of GalXC to therapeutic targets beyond the liver. Our research suggests that GalXC compounds developed to target hepatocytes in the liver are broadly applicable across multiple therapeutic areas, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. Our recent collaboration with Lilly provides both the opportunity to leverage our proprietary GalXC platform in order to generate new medicines for cardiometabolic diseases, and to target additional cell types to establish a presence in new therapeutic areas, including neurodegeneration and pain.
- Continue to invest in and extend our RNAi technology platform and intellectual property. We plan to continue to invest in expanding and improving our GalXC RNAi platform technology. We have a robust and growing patent portfolio that we believe covers our core technologies and our proprietary GalXC RNAi platform and other RNAi technologies. As of March 4, 2019, our worldwide patent estate, not including the patents and patent applications that we have licensed from third parties, included over 55 issued patents or allowed patent applications and at least 80 pending patent applications supporting commercial development of our RNAi molecules and delivery technologies.
- Leverage the experience and the expertise of our executive management team. To execute on our strategy, we have assembled an executive management team that has extensive experience in the biopharmaceutical industry. In addition, various members of our management team and our board of directors have contributed to the progress of the RNAi field through their substantial involvement in companies such as Cephalon Inc., Genta Inc., GlaxoSmithKline plc, Pfizer Inc., Sanofi S.A. (“Sanofi”), Sirna Therapeutics, Inc. (“Sirna”), and other companies. Our co-founder and chief executive officer, Douglas M. Fambrough III, Ph.D., was a lead venture capital investor and board member of Sirna, an early RNAi company acquired by Merck & Co., Inc. (“Merck”) in 2006 for $1.1 billion.

Our GalXC RNAi Technology Platform

The RNAi Therapeutic Modality

All of our GalXC drug discovery and development efforts are based on the therapeutic modality of RNAi, a highly potent and specific mechanism for silencing the activity of a targeted gene. In this naturally occurring biological process, double-stranded RNA molecules induce the enzymatic destruction of the mRNA of a target gene that contains sequences that are complementary to one strand of the therapeutic double-stranded RNA molecule. Our GalXC RNAi platform utilizes a proprietary structure of double-stranded RNA molecules configured for subcutaneous delivery to the liver. Due to the enzymatic nature of RNAi, a single GalXC molecule incorporated into the RNAi machinery can destroy hundreds or thousands of mRNAs from the targeted gene.

RNAi therapeutics represent a novel advance in drug development. Historically, the pharmaceutical industry has only developed small molecules or antibodies to inhibit the activity of disease-causing proteins. While this approach is effective for many diseases, many proteins cannot be inhibited by either small molecules or antibodies. Some proteins lack the binding pockets small molecules require for interaction. Other proteins are solely intracellular and therefore inaccessible to antibody-based therapeutics, which are limited to cell surface and extracellular proteins. The unique advantage of RNAi is that, instead of targeting proteins, RNAi silences the genes themselves via the targeted destruction of the mRNAs made from the gene. Rather than seeking to inhibit a protein directly, the RNAi approach is to prevent its creation in the first place.

We believe our approach to RNAi drug development provides the following qualities and has advantages compared to other therapeutic modalities:

- Our GalXC RNAi platform enables subcutaneous dosing for delivery to the liver. The GalXC RNAi platform is designed to enable convenient subcutaneous delivery for our emerging pipeline of liver-targeted RNAi investigational therapies. The GalXC RNAi platform does not involve lipid nanoparticles (“LNPs”) or other formulation components that facilitate drug delivery, which simplifies the platform and eliminates any requirement for functional excipients. Instead, our GalXC molecules are stabilized by chemical modifications and utilize a four base sequence known as a tetraloop, where each base is conjugated to a simple sugar, N-acetyl-D-galactosamine (“GalNAc”), that is specifically recognized by a receptor on the surface of hepatocyte liver cells. With the GalXC RNAi platform, a full human dose

may be administered via a single subcutaneous injection. After injection, the GalXC molecules enter the bloodstream and are exposed to the liver hepatocytes expressing the GalNAc receptor. After binding to the receptor, the GalXC molecules are internalized by the hepatocyte, ultimately enabling the GalXC molecules to access the RNAi machinery inside the hepatocyte.

Our GalXC molecules have a long duration of action. We believe our GalXC RNAi platform allows us to build a

- broad pipeline of therapeutics designed to have attractive pharmaceutical properties, including infrequent dosing (e.g., dosing

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that is on a monthly, quarterly, or an even less frequent basis) due to a long duration of action and higher potency of target gene silencing.

- Therapeutic opportunities beyond the liver. Our research suggests that GalXC compounds developed to target hepatocytes in the liver are broadly applicable across multiple therapeutic areas, including rare diseases, chronic liver diseases, cardiovascular diseases, and viral infectious diseases. Through our recent collaboration with Lilly, we intend to develop a new, non-hepatocyte platform, leveraging our proprietary GalXC platform, in order to generate new medicines for cardiometabolic diseases, and to target additional cell types to establish a presence in new fields, including neurodegeneration and pain.

Optimization of our GalXC molecules

For therapeutic use in humans, our GalXC molecules are optimized both with respect to base sequence and chemical modifications to increase stability and mask them from mechanisms that recognize foreign RNAs, in order to avoid inducing immune system stimulation. Our optimization process begins with an analysis of the target gene sequence using our proprietary GalXC prediction algorithm, which we developed based on the results of testing thousands of sequences for RNAi activity. We select the sequences with the highest predicted RNAi activity and apply patterns of chemical modification, including a GalNAc-linked tetraloop stem-loop structure, which designs in enhanced stability and hepatocyte delivery specificity and engineers out immunostimulatory activity. Our GalXC molecules routinely achieve high potencies, with EC50 values in the liver (i.e., the amount of material required to silence a target gene by 50 percent) typically in the 0.1 to 1.0 milligram per kilogram bodyweight (mg/kg) range in in vivo studies in mice. We have routinely generated GalXC molecules of this potency within 30 days of doing the initial algorithmic gene sequence analysis, which allows us to explore a large number of potential target genes when selecting our programs.

Development Focus

In choosing which development programs to internally advance, we apply the scientific, clinical, and commercial criteria listed below that we believe allow us to best leverage our GalXC RNAi platform and maximize value. We believe that our current development programs meet most or all of these criteria:

- Strength of therapeutic hypothesis. Our current and future product candidate gene targets are a well understood part of the disease process where a therapeutic intervention is likely to have substantial benefit for the patient.
- Readily-identified patient population. We seek disease indications where patients can be readily identified by the presence of characteristic genetic mutations or other readily-accessible disease features. In the case of genetic diseases, these are heritable genetic mutations that can be identified with available genetic tests.
- Predictivity of biomarkers for early efficacy assessment. We seek disease indications where there is a clear relationship between the disease status and an associated biomarker that we can readily measure. This approach will allow us to determine in early stages of clinical development whether our GalXC molecules are likely to have the expected biological and clinical effects in patients.
- High unmet medical need. We seek to provide patients with significant benefit and alleviation of disease. The indications we choose to approach have high unmet medical need, which is intended to enable us to better access patients and qualify for pricing and reimbursement that justify our development efforts.
- Rapid development path to approval. To reach commercialization expeditiously and to help ensure our ability to finance development of our product candidates, we have identified indications with the potential for rapid development through marketing approval. We believe that some of our product candidates have the potential to obtain breakthrough therapy designation as well as accelerated review process from the United States (“U.S.”) Food and Drug Administration (“FDA”).

We are focusing our efforts on three priority therapeutic programs that currently have a CTA filed, IND filed, or are in enabling studies in preparation to submit additional regulatory applications that will be necessary to initiate clinical trials. We are also focusing our efforts on a series of potential programs in the clinical candidate selection stage, or for which a provisional clinical candidate has been selected that may be elevated into IND/CTA enabling studies in the future, either on our own or in collaboration with larger pharmaceutical companies.

Our three priority programs are: DCR PHXC for the treatment of PH; DCR HBVS for the treatment of chronic HBV infection; and a program for an undisclosed rare disease. Our potential programs include additional rare disease programs, a program for the treatment of hypercholesterolemia, and multiple programs in various therapeutic areas

involving liver function.

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DCR PHXC for PH

We are developing DCR PHXC for the treatment of all types of PH. PH is a family of rare inborn errors of metabolism in which the liver produces excessive levels of oxalate, which in turn causes damage to the kidneys and to other tissues in the body. DCR PHXC is currently being investigated in a Phase 1 clinical trial called PHYOX. In non-clinical models of PH, DCR PHXC reduces oxalate production to near-normal levels, improving the disease condition.

PH encompasses three genetically distinct, autosomal-recessive, inborn errors of glyoxylate metabolism characterized by the over-production of oxalate, a highly insoluble metabolic end-product that is eliminated mainly by the kidneys. Patients with PH are predisposed to the development of multiple and recurrent urinary tract (urolithiasis) and kidney (nephrolithiasis) stones. Calculi formation is accompanied by nephrocalcinosis in some patients with PH (PH1 and some patients with PH2). This deposition of calcium oxalate in the renal parenchyma produces tubular toxicity and renal damage that is compounded by the effects of renal calculi related obstruction and frequent superimposed infections. Based on evaluation of genome sequence databases, there may be as many as 16,000 people with PH in the U.S. and major European countries.

PH1, PH2, and PH type 3 ("PH3"), are each characterized by a specific enzyme deficiency. PH1 is characterized by a deficiency of the liver peroxisomal enzyme alanine: glyoxylate-aminotransferase. Patients with PH1 represent approximately 80% of all patients currently diagnosed with PH. PH2 and PH3 are caused by dysfunction of glyoxylate reductase/hydroxypyruvate reductase and 4-hydroxy-2-oxoglutarate aldolase, respectively. Most patients are diagnosed in childhood or early adulthood. At present, no therapies are approved by regulatory authorities for the treatment of patients with PH. A number of supportive therapies are used in an attempt to mitigate some of the effects of the disease. Current medical management before renal failure develops is underpinned by hyperhydration with fluid intake recommendations of at least 3 liters per day per square meter of body-surface area (5 L/day for a 70-kg adult). These regimens can be problematic in infants and toddlers, necessitating placement of a gastrostomy tube to ensure adequate night time fluid administration. Affected patients are at considerable risk of serious complications during periods of increased fluid loss (fever, diarrhea/vomiting, and urinary tract infections) or when oral hydration is compromised (following surgical procedures). Oral potassium citrate administration is used to inhibit crystallization and alkalinize the urine. In PH1, an approximately 30% or greater reduction in urinary oxalate excretion may be achieved with oral vitamin B6 (Pyridoxine) administration at doses from 5 to 20 mg/kg in a small proportion of affected patients (10-20% of all PH1 patients).

For patients with more advanced disease, dialysis may be used in an attempt to remove endogenously over-produced oxalate. In contrast to 3 times weekly hemodialysis regimens more typically used in other types of renal failure, patients with PH may require hemodialysis 6 or 7 days per week. Given the limitations of dialysis and the inability to impact oxalate over-production substantially in most patients with PH1, most centers now consider liver transplantation approaches earlier in the disease course to minimize the risk of irreversible tissue damage. Current treatments include renal transplantation or, in PH1, combined liver and kidney transplantation. As with organ transplantation in other disease, these procedures are associated with significant medical risk and a requirement for long-term treatment with immunosuppressive drugs that are also associated with significant side effects.

We believe that there is a strong rationale for focusing our RNAi technology on the development of product candidates for the treatment of PH. DCR-PHXC, the Company's lead GalXC product candidate, was associated with normalization or near-normalization of urinary oxalate levels in a majority of adult patients with PH1 and PH2 following single-dose administration. As of a data cut on October 1, 2018, investigators reported that a single 3.0-mg/kg dose of DCR PHXC brought urinary oxalate levels into the normal range (defined as 24-hour excretion <0.460 mmol) at one or more post-dose time points in three out of four PH participants dosed at this level, including a mean maximal reduction in 24-hour urinary oxalate of 65% for the cohort, and a single 1.5-mg/kg dose led to near-normalization (defined as 24-hour excretion ≥0.460 to <0.600 mmol) in three out of four PH1 participants dosed at this level, which led to a mean maximal reduction in urinary oxalate of 50% in the five PH patients dosed at that level, including one PH2 patient. In addition, DCR-PHXC, as of the October 1, 2018 data cut, was safe and well-tolerated in this ongoing study based on data from 12 adult participants with PH1 (n=11) and PH2 (n=1) and 25 adult HVs.

LDHA reduction has a near-linear correlation with oxalate reduction and offers a minimal metabolic intervention. These benefits of LDHA inhibition may translate into consistent therapeutic activity even in the event of a missed dose. There are numerous case reports of LDHA deficiency naturally occurring in humans, with no reported adverse effects due to deficiency in the liver.

DCR HBVS for HBV

We have declared a GalXC RNAi platform-based product candidate, DCR HBVS, are conducting formal non-clinical development studies, and have initiated a Phase 1 clinical trial. We received CTA approval from the New Zealand Medicines and Medical Devices Safety Authority in November 2018 and ethics approval from the Health and Disability Ethics Committee in December 2018 for a Phase 1 clinical trial in healthy volunteers and patients with chronic HBV. The Phase 1 study was initiated in December 2018 and the first participants were dosed on January 24, 2019. We anticipate human POC data to be available in the fourth quarter of 2019. In addition, we filed for regulatory clearance in Australia, Hong Kong, South Korea, and Thailand in December 2018.

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Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of the hepatitis B surface antigen ("HBsAg") and sustained HBV deoxyribonucleic acid ("DNA") suppression in patient plasma or blood. DCR HBVS targets HBV messenger RNA and leads to greater than 99% reduction in circulated HBsAg in mouse models of HBV infection. Based on preclinical studies, and only if we receive appropriate regulatory approval to begin human clinical trials, we hope to determine the potential of DCR HBVS to reduce HBsAg expression and HBV DNA in HBV patients in a subcutaneous dosing paradigm.

According to the Hepatitis B Foundation, the World Health Organization estimates that globally, HBV is reported to be the most common serious liver infection with over 250 million patients chronically infected. Annual mortality directly linked to chronic HBV infection is estimated to be approximately 780,000 people with an estimated 650,000 of these deaths caused by cirrhosis and liver cancer as a result of chronic hepatitis B, and a further 130,000 of these deaths from complications associated with acute disease. Chronic HBV is characterized by the presence of the HBsAg for six months or more.

Nucleoside analogs and pegylated interferon regimens have been utilized to suppress the virus; however, while the regimens can offer long-term viral suppression if taken continuously, they do not provide a cure. The vast majority of treated patients do not achieve an immunological cure of chronic HBV infection under treatment with these agents. The chance of achieving a long-term immunological cure as measured by the clearance of HBsAg and sustained HBV DNA suppression may be possible with the introduction of novel drugs designed to reduce intrahepatic and serum HBsAg, as well as HBV DNA.

An undisclosed rare disease involving the liver

We are developing a GalXC-based therapeutic targeting a liver-expressed gene involved in a serious rare disease. For competitive reasons, we have not yet publicly disclosed the target gene or disease. We have selected this target gene and disease based on criteria that include having a strong therapeutic hypothesis, a readily-identifiable patient population, the availability of a potentially predictive biomarker, high unmet medical need, favorable competitive positioning and what we believe is a rapid projected path to approval. The disease is a genetic disorder where mutations in the disease gene lead to the production of an abnormal protein. The protein causes progressive liver damage and fibrosis, in some cases leading to cirrhosis and liver failure, and we believe that silencing of the disease gene will prevent production of the abnormal protein and thereby slow or stop progression of the liver fibrosis. Greater than 100,000 people in the U.S. are believed to be homozygous for the mutation that causes the liver disease, and at least 10% of those people, and potentially a significantly higher fraction, are believed to have liver-associated disease as a consequence. We intend to submit regulatory filings in the second quarter of 2019.

Additional pipeline programs

We have developed a robust portfolio of additional targets and diseases that we plan to pursue either on our own or in collaboration with partners. We have applied our GalXC technology to multiple gene targets across our disease focus areas of rare diseases, chronic liver diseases, and cardiovascular diseases. Pursuant to our strategy, we have established and continue to seek collaborations with larger, experienced pharmaceutical companies to advance our programs in the areas of chronic liver diseases and cardiovascular diseases, as well as select rare diseases that do not fit our criteria for a priority development program. The chronic liver and cardiovascular disease areas represent large and diverse patient populations, requiring complex clinical development and commercialization paths that we believe can be more effectively pursued in collaboration with larger pharmaceutical companies. For our additional rare diseases, we are continuing to assess their potential for clinical success and market opportunity while optimizing our GalXC molecules. For our additional pipeline programs, we may utilize more advanced versions of our GalXC technology that further improve pharmaceutical properties of the GalXC molecules, including enhancing the duration of action and potency. We have further optimized our GalXC technology platform, enabling the development of next generation GalXC molecules. Improvements to our GalXC compound include modification of the tetraloop end of the molecule, which can be applied to any target gene, resulting in a substantially longer duration of action and higher potency of target gene silencing in animal models across multiple targets. Modification of the tetraloop only impacts the passenger strand and does not impact the guide strand. These modifications are unique to our GalXC molecules and, we believe, provide a competitive advantage for us.

Other development programs

We also have developed a wholly-owned clinical candidate, DCR-BCAT, targeting the -catenin oncogene. DCR-BCAT is based on an extended version of our earlier generation non-GalXC RNAi technology and is delivered by our LNP tumor delivery system, EnCore™. We plan to out-license the DCR-BCAT opportunity, given our focus on our GalXC platform-based programs.

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The table below sets forth the state of development of our various GalXC RNAi platform product candidates as of March 4, 2019:

Status of Dicerna Programs

- Primary Hyperoxaluria. We are developing DCR PHXC for the treatment of all types of PH. Our Phase 1 clinical trial called PHYOX™ has completed dosing. PHYOX is a Phase 1 single ascending-dose study of DCR PHXC in healthy volunteers ("HVs") and study participants with PH. The study is divided into two groups:
  - Group A is a placebo-controlled, single-blind, single center study which enrolled 25 HVs.
  - Group B is an open-label, multi-center study enrolling up to 18 participants with PH type 1 ("PH1") or PH type 2 ("PH2").
  - The primary objective of the study is to evaluate the safety and tolerability of single of DCR PHXC in both groups.
  - The secondary objectives are to evaluate the pharmacodynamic effect of single doses of DCR PHXC on biochemical markers, and to characterize the pharmacokinetics of single doses of DCR PHXC in HVs and study participants with PH.

In May 2018, we dosed the first PH study participant with DCR PHXC in the Group B portion of the Phase 1 clinical trial and received notice from the U.S. FDA granting Orphan Drug Designation to DCR PHXC for treatment of PH. In August 2018, the European Medicines Agency ("EMA")'s Committee for Orphan Medicinal Products ("COMP") designated DCR PHXC as an orphan medicinal product for the treatment of PH in the European Union ("EU"). Currently, we are in the process of submitting requests for additional regulatory clearances necessary to commence clinical trials for our Phase 2 and Phase 3 studies in 2019. Key regulatory interactions with the FDA and EMA in anticipation of Phase 2 and Phase 3 studies are also underway.

We completed the Group A portion of the study in HVs and started the Group B portion of the study. Group B consists of participants with PH1 dosed at 1.5, 3.0, and 6.0-mg/kg, and participants with PH2 at a dose level of 1.5 and 3.0-mg/kg. As of January 17, 2019, we had dosed all 18 participants (15 PH1 participants and three PH2 participants). We reported interim results from the PHYOX trial on September 5, 2018 and presented updated results as of October 1, 2018 at Kidney Week in San Diego on October 25, 2018.

As of November 2018, three serious adverse events ("SAEs") have occurred in two participants (one subject experienced two discreet SAEs) in the PHYOX trial; none of these SAEs are related to the study intervention. There have been no clinically significant changes in electrocardiography, vital signs, laboratory, or hematology values. The investigators have observed in a total of 32 participants dosed (Group A and B together) mild-to-moderate injection site reactions in nine participants (28%), all of which were transient and resolved without intervention within 24 to 72 hours.

As of a data cut on October 1, 2018, investigators reported that a single 3.0-mg/kg dose of DCR PHXC brought urinary oxalate levels into the normal range (defined as 24-hour excretion <0.460 mmol) at one or more post-dose time points in three out of four PH participants dosed at this level, including a mean maximal reduction in 24-hour urinary oxalate of 65% for the cohort, and a single 1.5-mg/kg dose led to near-normalization (defined as 24-hour excretion ≥0.460 to <0.600

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mmol) in three out of four PH1 participants dosed at this level, and led to a mean maximal reduction in urinary oxalate of 50% in the five PH patients dosed at that level, including one PH2 patient.

Additionally, we intend to initiate a multi-dose study, which we hope will serve as a registration trial, in the first quarter of 2019, pending regulatory feedback.

- Chronic Hepatitis B Virus infection. We have declared a GalXC RNAi platform-based product candidate for the treatment of chronic HBV, DCR HBVS, and have initiated a Phase 1 clinical trial. We received CTA approval from the New Zealand Medicines and Medical Devices Safety Authority in November 2018 and ethics approval from the Health and Disability Ethics Committee in December 2018 for a Phase 1 clinical trial in HVs and patients with chronic HBV. The Phase 1 study was initiated in December 2018 and the first participant was dosed on January 24, 2019. We anticipate human POC data to be available in the fourth quarter of 2019. In addition, we filed for regulatory clearance in Australia, Hong Kong, South Korea, and Thailand in December 2018.

Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBsAg and sustained HBV DNA suppression in patient plasma or blood. DCR HBVS targets HBV mRNA and leads to greater than 99% reduction in circulated HBsAg in mouse models of HBV infection. DCR HBVS is comprised of a single GalXC molecule that targets HBV mRNAs within the HBsAg gene sequence region. In preclinical studies with a standard mouse model of HBV infection, we have found that targeting this region leads to superior HBsAg suppression, both in magnitude and duration of suppression, compared to targeting within the X gene sequence region. We believe that this difference in suppression derives from the role of the X gene product in indirectly regulating viral gene transcription such that the lack of X gene product leads to higher levels of viral gene transcription. Based on our preclinical studies, we hope to determine the potential of DCR HBVS to reduce HBsAg and HBV DNA levels in the blood of HBV patients in a commercially attractive subcutaneous dosing paradigm.

- An undisclosed rare disease involving the liver. We are developing a GalXC-based therapeutic, targeting a liver-expressed gene involved in a serious rare disease. For competitive reasons, we have not yet publicly disclosed the target gene or disease. The disease is a genetic disorder where mutations in the disease gene lead to the production of an abnormal protein. The protein causes progressive liver damage and fibrosis, in some cases leading to cirrhosis and liver failure, and we believe that silencing of the disease gene will prevent production of the abnormal protein and thereby slow or stop progression of the liver fibrosis. We intend to submit regulatory filings in the second quarter of 2019.

- Additional pipeline programs. We have developed a robust portfolio of additional targets and diseases that we plan to pursue either on our own or in collaboration with partners. We have applied our GalXC technology to multiple gene targets across our disease focus areas of rare diseases, chronic liver diseases, and cardiovascular diseases.

Pursuant to our strategy, we are seeking collaborations with larger and/or more experienced pharmaceutical companies to advance our programs in the areas of chronic liver diseases and cardiovascular diseases, as well as select rare diseases that do not fit our criteria for a priority development program. The chronic liver and cardiovascular disease areas represent large and diverse patient populations, requiring complex clinical development and commercialization paths that we believe can be more effectively pursued in collaboration with larger pharmaceutical companies. Certain rare diseases require complex clinical development and commercialization paths aligned with existing treatment paradigms that we believe can be more effectively pursued in collaboration with companies possessing certain rare disease expertise.

For our additional rare disease opportunities, we are continuing to assess their potential for clinical success and market opportunity while optimizing our GalXC molecules. For our additional pipeline programs, we may utilize more advanced versions of our GalXC technology that further improve pharmaceutical properties of the GalXC molecules, including enhancing the duration of action and potency. We have further optimized our GalXC technology platform, enabling the development of next generation GalXC molecules. Improvements to our GalXC compound include modification of the tetraloop end of the molecule, which can be applied to any target gene, resulting in a substantially longer duration of action and higher potency of target gene silencing in animal models across multiple targets. Modification of the tetraloop only impacts the sense strand and does not impact the antisense strand. These modifications are unique to our GalXC molecules and, we believe, provide a competitive advantage for the Company.

Partner Development Programs

Lilly Collaboration

On October 25, 2018, we entered into a Collaboration and License Agreement with Lilly (the "Lilly Collaboration Agreement"). The Lilly Collaboration Agreement is for the discovery, development, and commercialization of potential new medicines in the areas of cardiometabolic disease, neurodegeneration, and pain. Under the terms of the Lilly Collaboration Agreement, we and Lilly will seek to use our proprietary GalXC RNAi technology platform to progress new drug targets toward clinical development and commercialization. In addition, we will collaborate with Lilly to extend the GalXC RNAi platform technology to non-liver (i.e., non-hepatocyte) tissues, including neural tissues.

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The Lilly Collaboration Agreement provides that we will work exclusively with Lilly in the neurodegeneration and pain fields with the exception of mutually agreed upon orphan indications. Additionally, we will work exclusively with Lilly on select targets in the cardiometabolic field. Under the Lilly Collaboration Agreement, we will provide Lilly with exclusive and non-exclusive licenses to support the companies' activities and to enable Lilly to commercialize products derived from or containing compounds developed pursuant to such agreement. The Lilly Collaboration Agreement contemplates in excess of 10 targets.

Under the terms of the Lilly Collaboration Agreement, Lilly paid us a non-refundable, non-creditable upfront payment of $100.0 million, and made a concurrent stated $100.0 million equity investment in Dicerna at a premium pursuant to a share issuance agreement between the parties (the "Lilly Share Issuance Agreement"). Under the Lilly Collaboration Agreement, we are also eligible to potentially receive up to approximately $350.0 million per target in development and commercialization milestones, in addition to a $5.0 million payment due for each of the non-hepatocyte targets when a product achieves proof of principle in an animal model. In addition, the Lilly Collaboration Agreement also provides that Lilly will pay us mid-single to low-double digit royalties on product sales on a country-by-country and product-by-product basis until the later of expiration of patent rights in a country, the expiration of data or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement. Through December 31, 2018, no revenue has been recognized associated with the Lilly Collaboration Agreement.

Alexion Collaboration

On October 22, 2018, we and Alexion entered into a Collaborative Research and License Agreement (the "Alexion Collaboration Agreement") for the joint discovery and development of RNAi therapies for complement-mediated diseases. Under the terms of the Alexion Collaboration Agreement, we will collaborate with Alexion on the discovery and development of subcutaneously delivered GalXC candidates, currently in preclinical development, for the treatment of complement-mediated diseases with potential global commercialization by Alexion. We will lead the joint discovery and research efforts through the preclinical stage, and Alexion will lead development efforts beginning with Phase 1 studies. We will be responsible for manufacturing of the GalXC candidates through the completion of Phase 1, the costs of which will be paid by Alexion. Alexion will be solely responsible for the manufacturing of any product candidate subsequent to the completion of Phase 1.

The Alexion Collaboration Agreement also provides Alexion with exclusive worldwide licenses as well as development and commercial rights for two of our preclinical, subcutaneously delivered GalXC RNAi candidates and an exclusive option for the discovery and development of GalXC RNAi candidates against two additional complement pathway targets.

Under the terms of the Alexion Collaboration Agreement, Alexion paid us a non-refundable, non-reimbursable, and non-creditable upfront payment of $22.0 million, with Alexion Pharmaceuticals making a concurrent stated $15.0 million equity investment in Dicerna at a premium pursuant to a share issuance agreement between the parties (the "Alexion Share Issuance Agreement"). The Alexion Collaboration Agreement also provides for potential additional payments to us of up to $600.0 million from proceeds from target option exercises and development and sales milestones, as defined in the agreement, which is comprised of option exercise fees of up to $20.0 million, representing $10.0 million for each of the candidates selected; development milestones of up to $105.0 million for each product; and aggregate sales milestones of up to $160.0 million. Under the agreement, Alexion will also pay us mid-single to low-double digit royalties on potential product sales on a country-by-country, product-by-product basis until the later of the expiration of patent rights in a country, the expiration of market or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement. Through December 31, 2018, we recognized $0.1 million in revenue associated with the Alexion Collaboration Agreement.

BI Collaboration

On October 27, 2017, we entered into a Collaborative Research and License Agreement with BI (the "BI Agreement"), pursuant to which we and BI agreed to jointly research and develop product candidates for the treatment of chronic liver diseases, with an initial focus on nonalcoholic steatohepatitis ("NASH") using our GalXC platform. NASH is caused by the buildup of fat in the liver, potentially leading to liver fibrosis and cirrhosis. NASH has an especially

high prevalence among obese and diabetic patients and is an area of high unmet medical need. The BI Agreement is for the development of product candidates against an initial undisclosed target gene and includes an option for BI to add the development of product candidates that target a second gene (the "Additional Target"). We are working exclusively with BI to develop the product candidates against the undisclosed target gene. We are responsible for the discovery and initial profiling of the product candidates, including primary preclinical studies, synthesis, and delivery. BI is responsible for evaluating and selecting the product candidates for further development. If BI selects one or more product candidates, it will be responsible for further preclinical development, clinical development, manufacturing, and commercialization of those products. Pursuant to the BI Agreement, we granted BI a worldwide license in connection with the research and development of the product candidates and have transferred to BI certain intellectual property rights of the product candidates selected by BI for clinical development and commercialization. We also may provide assistance to BI in order to help BI further develop selected product candidates.

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Under the terms of the BI Agreement, BI agreed to pay us a non-refundable upfront payment of $10.0 million for the first target. During the term of the research program, we will be reimbursed by BI up to an agreed-upon limit for the cost of materials and third-party expenses that have been included in the preclinical studies. We are eligible to receive up to $191.0 million in potential development and commercial milestones related to the initial target. We are also eligible to receive royalty payments on potential global net sales, subject to certain adjustments, tiered from high single digits up to low double-digits.

In October 2018, BI exercised its option under the BI Agreement to add the development of product candidates targeting an Additional Target to the development activities governed by the BI Agreement. On December 31, 2018, we entered into an Additional Target Agreement (the "ATA") with BI. The ATA requires the parties to agree on a research work plan and budget for the Additional Target. The ATA also amends the BI Agreement to provide BI with the option to add the development of product candidates targeting a further additional undisclosed gene target for a three-year period, and to provide for the delivery of a replacement product candidate by us to BI in the event that a product candidate under the BI Agreement or the ATA fails at certain stages of preclinical or clinical development. Under the terms of the ATA, in accordance with the terms of the BI Agreement, and upon agreement of a research work plan and budget, BI agreed to pay us a non-refundable upfront payment of $5.0 million to exercise its initial option for development related to the Additional Target. Under the terms of the ATA, during the term of the research program, BI will reimburse us for certain expenses. We are eligible to receive up to $170.0 million in potential development and commercial milestones related to the Additional Target. We are also eligible to receive tiered royalty payments on potential global net sales, subject to certain adjustments, in the mid-single digits. Other than as set forth in the ATA, development of the Additional Target will be subject to the terms of the BI Agreement. Under the ATA, if BI elects to exercise the second option, the parties would agree to a research work plan and budget for the additional gene and negotiate development and commercialization milestones and royalty payments to Dicerna. BI would make another option fee payment to us of $5.0 million. Through December 31, 2018, we recognized $7.1 million in revenue associated with the BI Agreement.

License Agreements

In December 2014, we licensed all of our non-U.S. intellectual property rights to a non-U.S. wholly-owned subsidiary. In December 2015, we licensed our U.S. intellectual property rights to the same non-U.S. wholly-owned subsidiary. In December 2016, the same non-U.S. wholly-owned subsidiary distributed the U.S. intellectual property rights back to us. In 2017, we amended a licensing agreement with the non-U.S. subsidiary to exclude from the scope and operation of that agreement the intellectual property licensed to BI pursuant to the BI Agreement. As such, effective October 27, 2017 certain rights associated with the BI Agreement reverted to us.

Intellectual Property

We are seeking multifaceted and multi-layered protection for our intellectual property that includes licenses, confidentiality and non-disclosure agreements, copyrights, patents, trademarks, and trade secrets. We enter into confidentiality and proprietary rights agreements with our employees, consultants, collaborators, subcontractors, and other third parties and generally seek to control access to our documentation and proprietary information.

Patents and proprietary rights

We own U.S. patents and a number of pending patent applications with claims to methods and compositions of matter that cover various aspects of our RNAi technology and our discovery technologies, including our proprietary GalXC technology. These U.S. patents include: U.S. 8,349,809 (issued in January 2013, with a projected expiration date of January 2030); U.S. 8,513,207 (issued in August 2013, with a projected expiration date of May 2030); and U.S. 8,927,705 (issued in January 2015, with a projected expiration date of July 2030). We also own numerous patents and patent applications covering specific RNAi sequences that drive activity against 12 high value disease targets, including targets for our key programs. We have issued or pending claims to RNAi molecules, pharmaceutical compositions/formulations, methods of use, including in vitro and in vivo methods of reducing target gene expression, methods of treatment, methods of inhibiting cell growth, and methods of synthesis.

Our strategy around protection of our proprietary technology, including any innovations and improvements, is to obtain patent coverage in various jurisdictions around the world with a focus on jurisdictions that represent significant

global pharmaceutical markets. Generally, patents have a term of 20 years from the earliest non-provisional priority date, assuming that all maintenance fees are paid, no portion of the patent has been terminally disclaimed and the patent in question has not been invalidated by a court with proper jurisdiction. In certain jurisdictions, and in certain circumstances, patent terms can be extended or shortened. We are obtaining worldwide patent protection for at least novel molecules, composition of matter, pharmaceutical formulations, methods of use, including treatment of disease, methods of manufacture and other novel uses for the inventive molecules originating from our research and development efforts. We continuously assess whether it is strategically more favorable to maintain confidentiality for the “know-

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how" regarding a novel invention or the trade secrets that may be inherent in a given process or method rather than pursue patent protection. For each patent application that is filed we strategically tailor our claims in accordance with the existing patent landscape around a particular technology.

We cannot predict with any certainty if any third-party U.S. or foreign patent rights, other proprietary rights, will be deemed infringed by the use of our technology. Nor can we predict with certainty which, if any, of these rights will or may be asserted against us by third parties. Should we need to defend ourselves and our collaborators against any such claims, substantial costs may be incurred. Furthermore, parties making such claims may be able to obtain injunctive or other equitable relief, which could effectively block our ability to develop or commercialize some or all of our products in the U.S. and abroad and could result in the award of substantial damages. In the event of a claim of infringement, we or our collaborators may be required to obtain one or more licenses from a third party. There can be no assurance that we can obtain a license on a reasonable basis should we deem it necessary to obtain rights to an alternative technology that meets our needs. The failure to obtain a license may have a material adverse effect on our business, results of operations and financial condition.

We also rely on trade secret protection for our confidential and proprietary information. No assurance can be given that we can meaningfully protect our trade secrets on a continuing basis. Others may independently develop substantially equivalent confidential and proprietary information or otherwise gain access to our trade secrets.

It is our policy to require our employees and consultants, outside scientific collaborators, sponsored researchers, and other advisors who receive confidential information from us, to execute confidentiality agreements upon the commencement of employment or consulting relationships. These agreements provide that all confidential information developed or made known to these individuals during the course of the individual's relationship with us is to be kept confidential and is not to be disclosed to third parties except in specific circumstances. The agreements provide that all inventions conceived by an employee shall be our property. There can be no assurance, however, that these agreements will provide meaningful protection or adequate remedies for our trade secrets in the event of unauthorized use or disclosure of such information.

Our success will depend in part on our ability to obtain and maintain patent protection, preserve trade secrets, prevent third parties from infringing upon our proprietary rights, and operate without infringing upon the proprietary rights of others, both in the U.S. and other territories worldwide.

See Item 1A – "Risk Factors – Risks Related to Intellectual Property" for a more detailed discussion of the risks to our intellectual property.

Competition

To our knowledge, there are no other companies developing GalXC molecules for therapeutic use. We believe that our scientific knowledge and expertise in RNAi-based therapies provide us with competitive advantages over the various companies and other entities that are attempting to develop similar treatments. However, many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals, and marketing approved products than we do. Our competition can be grouped into three broad categories:

- Other companies working to develop RNAi therapeutic products;
- Companies developing technology known as antisense, which, similar to the RNAi therapy we use, attempts to silence specific genes; and
- Commercialized products and product candidates, as well as development programs that treat the same diseases for which we are also developing treatments.

Our success will be based, in part, upon our ability to identify, develop, and manage a portfolio of drugs that offer competitive advantages such as improved safety, more convenient dosing, and higher efficacy competing products for the treatment of our targeted patients. Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are superior to the products we may develop.

Summarized below is information on perceived competition for our most advanced product candidates.

Primary Hyperoxaluria

Currently, there are no approved drugs to treat primary hyperoxaluria. The current standard of care for treating PH1 is a dual-organ transplant, specifically a kidney and liver transplant in patients with PH1, which is often difficult to

perform due to lack of donors and the threat of organ rejection. Other treatments include pyridoxine regimens and intensive dialysis, as well as treatments

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generally used in kidney stone disorders such as high-volume fluid intake and oral citrate. These other treatments do not halt disease progression.

We believe that the following product candidates, if approved, could compete with DCR-PHXC:

Company	Drug	Drug Description	Phase
Alnylam Pharmaceuticals, Inc.	Lumasiran (formerly ALN-GO1)	RNAi therapeutic targeting glycolate oxidase	Phase 3
Oxthera AB	Oxabact	Bacteria intended to interact with the intestinal epithelial cells and promote secretion of oxalate from the body	Phase 3
Allena Pharmaceuticals, Inc.	Reloxaliase (formerly ALLN-177)	RNAi enzyme to reduce oxalate levels	Phase 2

There are also other companies that have preclinical development programs for the potential treatment of PH, such as Intellia Therapeutics, Inc.

Hepatitis B Virus

Nucleoside analogs and pegylated interferon regimens have been utilized in order to suppress the hepatitis B virus in patients with chronic HBV infection. However, while these regimens can offer long-term viral suppression, they do not provide a cure, and they usually require lifelong therapy. A finite treatment option with the chance of achieving a long-term immunological cure, as measured by the clearance of HBsAg and sustained HBV DNA suppression, may be possible with the introduction of novel drugs designed to reduce intrahepatic and serum HBsAg, as well as HBV DNA.

We believe that the following product candidates, if approved, could compete with DCR-HBVS:

Company	Drug	Drug Description	Phase
Alnylam Pharmaceuticals, Inc. Partner: Vir Biotechnology	ALN-HBV02	RNAi-GalNAc conjugate	Phase 1/2
Arbutus Biopharma	ARB-1467	RNAi lipid nanoparticle formulation	Phase 2
Arbutus Biopharma	AB-729	RNAi-GalNAc conjugate	Preclinical
Arrowhead Pharmaceuticals, Inc. Partner: Janssen	ARO-HBV	RNAi-GalNAc conjugate	Phase 1
Ionis Pharmaceuticals, Inc. Partner: GlaxoSmithKline	IONIS-HBVRx	RNA-targeted antisense technology	Phase 2
Ionis Pharmaceuticals, Inc. Partner: GlaxoSmithKline	IONIS-HBV-L_{Rx}	Antisense oligonucleotide-GalNAc conjugate	Phase 2
F. Hoffman-La Roche, Ltd	RG6004	Antisense oligonucleotide-GalNAc conjugate	Phase 1

There are also other companies that have preclinical development programs for the potential treatment of HBV.

If our lead product candidates are approved for the indications for which we undertake clinical trials, they may compete with therapies that are either in development or currently marketed by our competitors. However, notwithstanding the availability of existing drugs or drug candidates, we believe sufficient unmet medical need exists to warrant the continuing advancement of our investigational RNAi therapeutic programs.

Sales and Marketing

Our current focus is on the development of our existing portfolio, the initiation and completion of clinical trials, and, where appropriate, the registration of our product candidates. We currently do not have marketing, sales, or distribution capabilities. If we receive marketing and commercialization approval for any of our product candidates, we intend to market the product either directly or through strategic alliances and distribution agreements with third parties. The ultimate implementation of our strategy for realizing the financial value of our product candidates is dependent on the results of clinical trials for our product candidates, the availability of funds, our ability to obtain adequate coverage of and reimbursement for our products, compliance with laws governing our sales and marketing activities, and the ability to negotiate acceptable commercial terms with third parties.

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Manufacturing and Supply

We do not currently own or operate any manufacturing facilities for the production of preclinical, clinical, or commercial quantities of any of our product candidates. For each product candidate, we currently contract with third-party manufacturers and suppliers for certain drug materials, and we expect to continue to do so to meet the preclinical and clinical requirements of our product candidates.

Presently, some of the drug starting materials for our manufacturing activities are supplied by a single source supplier and we are in the process of identifying secondary suppliers. We believe that adequate alternative sources for such supplies exist; however, there is a risk that, if supplies are interrupted, it would materially harm our business. We typically order raw materials and services on a purchase order basis and do not enter into long-term dedicated capacity or minimum supply arrangements.

Manufacturing is subject to extensive regulations that impose various procedural and documentation requirements, which govern record-keeping, manufacturing processes and controls, personnel, quality control, and quality assurance, among others. Our contract manufacturing organizations manufacture our product candidates under current Good Manufacturing Practice ("cGMP") conditions. cGMP is a regulatory standard for the production of pharmaceuticals that will be used in humans.

Government Regulation and Product Approval

Governmental authorities in the U.S., at the federal, state and local level, and other countries extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, promotion, storage, record-keeping, advertising, distribution, sampling, pricing, sales and marketing, safety, post-approval monitoring and reporting, and export and import of products such as those we are developing. Our product candidates must be approved by the FDA through the NDA process before they may be legally marketed in the U.S. and will be subject to similar requirements in other countries prior to marketing in those countries. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and the extensive laws and regulations that apply to drug products and product candidates in the U.S. are subject to change.

U.S. government regulation

NDA approval processes

In the U.S., the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act (the "FDCA") and implementing regulations. Failure to comply with the applicable U.S. requirements at any time during the product development or approval process, or after approval, may result in a delay of approval or subject an applicant to administrative or judicial sanctions, any of which could have a material adverse effect on us. These sanctions could include:

- refusal to approve pending applications;
- withdrawal of an approval;
- imposition of a clinical hold;
- issuance of warning or untitled letters;
- product recalls;
- product seizures;
- refusals of government contracts;
- total or partial suspension of production or distribution; or
- injunctions, fines, restitution, disgorgement, civil penalties or criminal prosecution.

The process required by the FDA before a drug may be marketed in the U.S. generally includes the following:

- completion of non-clinical laboratory tests, animal studies, and formulation studies conducted according to Good Laboratory Practices ("GLPs") or other applicable laws and regulations;
- submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- approval by an institutional review board ("IRB") at each clinical site before each trial may be initiated
- performance and inspection of adequate and well-controlled human clinical trials and clinical data according to FDA regulations and Good Clinical Practices ("GCP") to establish the safety and efficacy of the product candidate for its intended use;

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- submission of an NDA to the FDA and the FDA's acceptance of the NDA for filing;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product candidate is produced to assess compliance with cGMP to assure that the facilities, methods, and controls are adequate to preserve the product candidate's identity, strength, quality, and purity;
- satisfactory completion of an FDA inspection of the major investigational sites to ensure data integrity and assess compliance with GCP requirements; and
- FDA review and approval of the NDA.

Once a pharmaceutical candidate is identified for development, it enters the preclinical or non-clinical testing stage. non-clinical tests include laboratory evaluations of product chemistry, stability, toxicity and formulation, as well as animal studies. An IND sponsor must submit the results of the non-clinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. Some non-clinical testing may continue even after the IND is submitted. In addition to including the results of the non-clinical studies, manufacturing, and quality information, the IND will also include a protocol detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated if the first phase lends itself to an efficacy determination. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, places the IND on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can begin. A clinical hold may occur at any time during the life of an IND and may affect one or more specific studies or all studies conducted under the IND.

All clinical trials must be conducted under the supervision of one or more qualified investigators in accordance with FDA regulations and GCP. They must be conducted under protocols detailing the objectives of the trial, dosing procedures, research subject selection and exclusion criteria and the safety and effectiveness criteria to be evaluated. Each protocol and protocol amendments must be submitted to the FDA as part of the IND, and progress reports detailing the status of the clinical trials must be submitted to the FDA annually. Sponsors also must timely report to the FDA serious and unexpected adverse reactions, any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigation brochure or any findings from other studies or animal or in vitro testing that suggest a significant risk in humans exposed to the drug. All research subjects or their legally authorized representatives must provide their informed consent in writing prior to their participation in a clinical trial. An IRB at each institution participating in the clinical trial must review and approve the protocol and the informed consent form before a clinical trial commences at that institution, monitor the study until completed and otherwise comply with IRB regulations. Information about most clinical trials must be submitted within specific timeframes to the National Institutes of Health ("NIH") to be publicly posted on the ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined.

- Phase 1 – The product candidate is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, and elimination. In the case of some product candidates for severe or life-threatening diseases, such as cancer, especially when the product candidate may be inherently too toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- Phase 2 – Clinical trials are performed on a limited patient population intended to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- Phase 3 – Clinical trials are undertaken to further evaluate dosage, clinical efficacy, and safety in an expanded patient population at geographically dispersed clinical study sites. These studies are intended to establish the overall risk-benefit ratio of the product and provide an adequate basis for product labeling.

Human clinical trials are inherently uncertain, and Phase 1, Phase 2, and Phase 3 testing may not be successfully completed. The FDA, the sponsor, or a data safety monitoring board, may suspend a clinical trial at any time for a variety of reasons, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product candidate has been associated with unexpected serious harm to patients.

During the development of a new product candidate, sponsors are given opportunities to meet with the FDA at certain points prior to the submission of an IND, at the end of Phase 2 and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date and for the FDA to provide advice on the next phase of development. Sponsors typically use the meeting at the end of Phase 2 to discuss their Phase 2 clinical results and present their plans for the pivotal Phase 3 clinical trial that they believe will support the approval of an NDA. If a Phase 2 clinical trial is the subject of discussion at the end of Phase 2 meeting with the FDA, a sponsor may be able to request a

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Special Protocol Assessment, the purpose of which is to reach agreement with the FDA on the Phase 3 clinical trial protocol design and analysis that will form the primary basis of an efficacy claim.

Concurrent with clinical trials, sponsors usually complete additional animal safety studies and also develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing commercial quantities of the product candidate in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and the manufacturer must develop methods for testing the safety, identity, strength, purity, and quality of the product candidate. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its proposed shelf-life.

Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured and tested and will not approve the product unless cGMP compliance is satisfactory. The FDA will also typically inspect one or more clinical sites to assure compliance with FDA regulations and GCP.

The results of product development, non-clinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests and other control mechanisms, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA is subject to the payment of user fees, but a waiver of such fees may be obtained under specified circumstances. The FDA reviews all NDAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. It may request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing.

Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use and whether its manufacturing is cGMP-compliant. The FDA typically requires that an NDA include data from two adequate and well-controlled clinical trials, but approval may be based upon a single adequate and well-controlled clinical trial in certain circumstances. The FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical or other data. Even if such data are submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. The FDA may refer the NDA to an advisory committee for review and recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may condition approval on the completion of post approval studies. Such studies may involve clinical trials designed to further assess a product’s safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized. If the FDA determines that it is necessary to ensure the safe use of the drug, the FDA may also condition approval on the implementation of a risk evaluation and mitigation strategy (“REMS”). The REMS could include medication guides, physician communication plans or elements to assure safe use, such as restricted distribution methods, patient registries, or other risk minimization tools.

Expedited review and approval

The FDA has various programs, including Fast Track, priority review, breakthrough, and accelerated approval, which are intended to expedite or simplify the process for reviewing product candidates. Generally, product candidates that are eligible for these programs are those for serious or life-threatening conditions, those with the potential to address unmet medical needs and those that offer meaningful benefits over existing treatments. A sponsor can request application of these programs either alone or in combination with each other, depending on the circumstances. Even if a product candidate qualifies for one or more of these programs, the FDA may later decide that the product candidate no longer meets the conditions for qualification or that the time period for FDA review or approval will be shortened. None of the expedited approval programs change the NDA approval standard applied to a product.

New drugs are eligible for Fast Track status 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 status entitles such a drug to expedited review and frequent contact with the FDA review division. Unlike other expedited review programs, Fast Track designation allows the FDA to accept for review individual sections of the NDA on a rolling basis. The FDA may also grant a priority review designation to drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists. A priority review means that the goal for the FDA to review an application is six months from filing of an NDA, rather than the standard review of ten months from filing under current Prescription Drug User Fee Act guidelines. Most products that are eligible for Fast Track designation are also likely to be considered appropriate to receive a priority review.

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Drug products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA typically requires that a sponsor of a product candidate receiving accelerated approval conduct post-approval clinical trials. As an additional condition of approval, the FDA currently requires pre-approval of all promotional materials, which could adversely impact the timing of the commercial launch of the product.

The FDA may expedite the approval of a designated breakthrough therapy, which is a drug that is intended to treat a serious or life-threatening disease or condition for which 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. A sponsor may request that a drug be designated as a breakthrough therapy at any time during the clinical development of the product. If the FDA designates a drug as a breakthrough therapy, the FDA must take the appropriate steps to expedite the development and review of the application, which may include holding meetings with the sponsor and the review team throughout the development of the drug; providing timely advice to the sponsor regarding the development of the drug to ensure that the development program is as efficient as practicable; involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review; and taking steps to ensure that the design of the clinical trials is as efficient as practicable.

In December 2016, the 21st Century Cures Act (“Cures Act”), was signed into law. The Cures Act included numerous provisions that may be relevant to our product candidates, including provisions designed to speed development of innovative and breakthrough therapies. The Cures Act amends the FDCA and the Public Health Service Act, to reauthorize and expand funding for the NIH and to authorize the FDA to increase spending on innovation projects. Central to the Cures Act are provisions that enhance and accelerate the FDA’s processes for reviewing and approving new drugs and supplements to approved NDAs. The Cures Act also includes a provision that requires certain manufacturers or distributors of an investigational drug to make their policies on the availability of certain expanded access programs publicly available. Because the Cures Act was enacted relatively recently and the FDA may take several years to develop these policies, it is difficult to know the full extent of how the Cures Act will affect our business.

Patent term restoration and marketing exclusivity

Depending upon the timing, duration and specifics of FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product candidate’s approval date. The patent term restoration period is generally one half of the time between the effective date of an IND and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved product candidate is eligible for the extension and the application for extension must be made prior to expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration date, depending on the expected length of clinical trials and other factors involved in the submission of the relevant NDA.

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the U.S. to the first applicant to gain approval of an NDA for a new chemical entity. A product candidate is a new chemical entity if the FDA has not previously approved any other new product candidate containing the same active moiety, which is the molecule or ion responsible for the action of the product candidate substance. During the exclusivity period, the FDA may not accept

for review an abbreviated new drug application ("ANDA") or a 505(b)(2) NDA (i.e., an NDA that contains full safety and effectiveness reports but allows at least some of the information required for NDA approval to come from studies not conducted by or for the applicant) submitted by another company for another version of such product candidate where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an approved NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing product candidate. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for product candidates containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right

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of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Orphan drug designation

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

If a product candidate that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product candidate is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications including a full NDA to market the same product candidate for the same indication, except in very limited circumstances, for seven years. Orphan drug exclusivity, however, could also block the approval of one of our product candidates for seven years if a competitor obtains approval of the same product candidate as defined by the FDA prior to us.

On August 8, 2017, the FDA Reauthorization Act of 2017 (“FDARA”) was enacted. FDARA, among other things, codified the FDA’s pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act, including the FDARA amendment, its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

Pediatric exclusivity, pediatric use and rare pediatric disease priority review vouchers

Under the Best Pharmaceuticals for Children Act, certain product candidates may obtain an additional six months of exclusivity if the sponsor submits information requested in writing by the FDA (a “Written Request”) relating to the use of the active moiety of the product candidate in children. The FDA may not issue a Written Request for studies on unapproved or approved indications or where it determines that information relating to the use of a product candidate in a pediatric population, or part of the pediatric population, may not produce health benefits in that population. FDARA amended the FDCA to provide that a drug, for which an application has been submitted or approved pursuant to section 505(b)(2) or 505(j) of the FDCA, will not be considered ineligible for approval or misbranded because the labeling of such drug omits a pediatric indication or other pediatric labeling information when the omitted pediatric information is protected by patent or marketing exclusivity. FDARA further permits FDA to require specific labeling for such products related to the omitted pediatric indication and information to, among other things, make clear that the omission of the information is related to the exclusivity. We do not know if or how such changes to the pediatric exclusivity provisions might affect our business.

In addition, the Pediatric Research Equity Act (“PREA”) requires a sponsor to conduct pediatric studies for most product candidates and biologics, for a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration. Under PREA, original NDAs, biologics license applications and supplements thereto must contain a pediatric assessment unless the sponsor has received a deferral or waiver. The required assessment must assess the safety and effectiveness of the product candidate for the claimed indications in all relevant pediatric subpopulations and support dosing and administration for each pediatric subpopulation for which the product candidate is safe and effective. The sponsor or the FDA may request a deferral of pediatric studies for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the product candidate or biologic is ready for approval for use in adults before pediatric studies are complete or that additional safety or effectiveness data needs to be collected before the pediatric studies begin. After April 2013, the FDA must

send a noncompliance letter to any sponsor that fails to submit the required assessment, keep a deferral current or fails to submit a request for approval of a pediatric formulation. PREA does not apply to any drug for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s).

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Section 529 of the FDCA is intended to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Although there are existing incentive programs to encourage the development and study of drugs for rare diseases, pediatric populations, and unmet medical needs, section 529 provides an additional incentive for rare pediatric diseases, which may be used alone or in combination with other incentive programs. "Rare pediatric disease" is defined as a disease that:

- "primarily affects individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents," which is interpreted as meaning that greater than 50% of the affected population in the U.S. is aged 0 through 18 years; and
- is "a rare disease or condition" as defined in the FDCA, which includes diseases and conditions that affect fewer than 200,000 persons in the U.S. and diseases and conditions that affect a larger number of persons and for which there is no reasonable expectation that the costs of developing and making available the drug in the U.S. can be recovered from sales of the drug in the U.S.

Under section 529, the sponsor of a human drug application for a rare pediatric disease drug product may be eligible for a voucher that can be used (or sold) to obtain a priority review for a subsequent human drug application submitted under section 505(b)(1) of the FDCA or section 351 of the Public Health Service Act after the date of approval of the rare pediatric disease drug product. The rare pediatric disease priority review vouchers program was re-authorized by Congress in the Cures Act, extending the program through 2020. The FDA has issued draft Guidance for Industry for Rare Pediatric Disease Priority Review Vouchers.

Post-approval requirements

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements is not maintained or if problems occur after the product candidate reaches the market. Requirements for additional Phase 4 trials (post-approval marketing studies) to confirm safety and efficacy may be imposed as a condition of approval. Later discovery of previously unknown problems with a product candidate may result in REMS or even complete withdrawal of the product candidate from the market. After approval, some types of changes to the approved product candidate, such as adding new indications, manufacturing changes and additional labeling changes, are subject to further FDA review and approval. In addition, the FDA may require testing and surveillance programs to monitor the effect of approved product candidates that have been commercialized, and the FDA has the power to prevent or limit further marketing of a product candidate based on the results of these post-marketing programs.

Any product candidates manufactured or distributed by us pursuant to FDA approvals are subject to continuing regulation by the FDA, including, among other things:

- record-keeping requirements;
- reporting of adverse experiences with the product candidate;
- submission of periodic reports;
- providing the FDA with updated safety and efficacy information;
- drug sampling, stability and distribution requirements;
- notifying the FDA and gaining its approval of specified manufacturing or labeling changes; and
- complying with statutory and regulatory requirements for promotion and advertising.

Drug manufacturers and other entities involved in the manufacture and distribution of approved product candidates are required to register their establishments and provide product listing information to the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and some state agencies for compliance with cGMP and other laws.

Regulation outside of the U.S.

In addition to regulations in the U.S., we will be subject to regulations of other jurisdictions governing any clinical trials and commercial sales and distribution of our product candidates. Whether or not we obtain FDA approval for a product, we must obtain approval by the comparable regulatory authorities of countries outside of the U.S. before we can commence clinical trials in such countries, and approval of the regulators of such countries or supranational areas, such as the European Union ("EU"), before we may market products in those countries or areas. The approval process and requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from place to place, and the time may be longer or shorter than that required for FDA approval.

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Under EU regulatory systems, a company may submit marketing authorization applications either under a centralized or decentralized procedure. The centralized procedure, which is compulsory for certain medicines, including those produced by biotechnology or those intended to treat HIV, AIDS, cancer, neurodegenerative disorders, autoimmune and other immune dysfunctions, viral diseases or diabetes and is optional for those medicines which are a significant therapeutic, scientific or technical innovation or whose authorization would be in the interest of public health, provides for the grant of a single marketing authorization that is valid for all EU member states. Through the decentralized procedure, a medicinal product that has not yet been authorized in the EU can be simultaneously authorized in several EU member states. The mutual recognition procedure provides for mutual recognition of national approval decisions. Under this procedure, the holder of a national marketing authorization may submit an application to the remaining member states. Within 90 days of receiving the applications and assessment reports, each member state must decide whether to recognize the approval. If a member state does not recognize the marketing authorization, the disputed points are eventually referred to the European Commission, whose decision is binding on all member states.

As in the U.S., we may apply for designation of a product candidate as an orphan drug for the treatment of a specific indication in the EU before the application for marketing authorization is made. Sponsors of orphan drugs in the EU can enjoy economic and marketing benefits, including up to 10 years of market exclusivity for the approved indication. During such period, marketing authorization applications for "similar" medicinal products will not be accepted, unless another applicant can show that its product is safer, more effective or otherwise clinically superior to the orphan-designated product. In the EU, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication.

Coverage and Reimbursement

Sales of our products will depend, in part, on the extent to which the costs of our products will be covered and paid for by third-party payors, such as government health programs, commercial insurance and managed healthcare organizations. Third-party payors may limit coverage to specific products on an approved list or formulary, which might not include all of the FDA-approved products for a particular indication. Also, third-party payors may refuse to include a particular branded drug on their formularies or otherwise restrict patient access to a branded drug when a less costly generic equivalent or another alternative is available. Third-party payors are 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, restrictions on reimbursement and requirements for substitution of generic products. For example, the current U.S. administration has indicated support for possible new measures to regulate drug pricing. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could significantly limit our net revenue and financial 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 after approved as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products on a profitable basis.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 ("MMA") imposed new requirements for the distribution and pricing of prescription drugs for Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities which will provide coverage of outpatient prescription drugs. Part D plans include both stand-alone prescription drug benefit plans and prescription drug coverage as a supplement to Medicare Advantage plans. Unlike Medicare Part A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for our products for which we receive marketing approval. However, any

negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payors.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act of 2010, collectively referred to as the ACA, enacted in March 2010, has had a significant impact on the health care industry by, for example, expanding coverage for the uninsured and seeking to contain overall healthcare costs. Regarding pharmaceutical products, among other things, the ACA contains provisions that may reduce the profitability of drug products such as expanding and increasing industry rebates for drugs covered under Medicaid programs and making changes to the coverage requirements under the Medicare Part D program. Recently, the current U.S. administration and U.S. Congress have expressed a desire to modify, repeal, or otherwise invalidate all, or certain provisions of, the ACA, which has contributed to the uncertainty of the ongoing implementation and impact of the ACA and also underscores the potential for additional health care reform going forward. For example, the newly enacted federal

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income tax law includes a provision effective January 1, 2019, repealing the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." Congress may consider other legislation that would alter other aspects of the ACA. In addition, at least one U.S. District Court has ruled that the ACA is unconstitutional, and while the ruling was stayed pending appellate review, it is uncertain whether the ruling will be affirmed, and if affirmed, whether all or some of the ACA would then survive.

There is still uncertainty with respect to the impact the current U.S. administration, the U.S. Congress, and the courts may have, if any, and any changes will likely take time to unfold. Such reforms could have an adverse effect on anticipated revenues from product candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop product candidates.

Further legislation or regulation could be passed that could harm our business, financial condition and results of operations. Other legislative changes have been proposed and adopted since the ACA was enacted. For example, in August 2011, President Obama 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 on Deficit Reduction did not achieve a targeted deficit reduction of at least $1.2 trillion for fiscal years 2012 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and will stay in effect through 2027 unless additional Congressional action is taken. In addition, on February 9, 2018, Congress passed the Bipartisan Budget Act that made several healthcare reforms. For example, the law changes the discounts manufacturers are required to apply to their drugs under the Coverage Gap Discount Program from 50% to 70% of the negotiated price starting in 2019. In addition, the law increases civil and criminal penalties for fraud and abuse laws, including, for example, criminal fines for violations of the Anti-Kickback Statute increase from $25,000 to $100,000 and corresponding prison sentences also increase from no more than five years to no more than ten years.

Also, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed products, which have resulted in several Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. Individual states in the United States have also become increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures. For example, in September 2017, the California State Assembly approved SB17 which requires pharmaceutical companies to notify health insurers and government health plans at least 60 days before any scheduled increases in the prices of their products if they exceed 16% over a two-year period, and further requiring pharmaceutical companies to explain the reasons for such increases. Effective in 2016, Vermont passed a law requiring certain manufacturer identified by the state to justify their price increases.

In addition, in some non-U.S. jurisdictions, the proposed pricing for a product candidate must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls and/or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our product candidates. Historically, product candidates launched in the EU do not follow price structures of the U.S. and generally tend to have price structures that are significantly lower.

Other Healthcare Fraud and Laws

In the U.S., our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including but not limited to, the Centers for Medicare and Medicaid Services ("CMS"), other divisions of

the U.S. Department of Health and Human Services (such as the Office of Inspector General and the Health Resources and Service Administration), the U.S. Department of Justice (the "DOJ") and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs may have to comply with the anti-fraud and abuse provisions of the Social Security Act, the false claims laws, the privacy and security provisions of the Health Insurance Portability and Accountability Act ("HIPAA") and similar state laws, each as amended, as applicable.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any item or service reimbursable, in whole or in part, under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include

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anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between therapeutic product manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending 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 of its facts and circumstances. Additionally, the intent standard under the Anti-Kickback Statute was amended by the ACA to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the ACA codified case law 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 (the "FCA") (discussed below).

Additionally, on January 31, 2019, the Department of Health and Human Services ("HHS") and HHS Office of Inspector General ("OIG") proposed an amendment to one of the existing Anti-Kickback safe harbors (42 C.F.R. 1001.952(h)) which would prohibit certain pharmaceutical manufacturers from offering rebates to pharmacy benefit managers ("PBMs") in the Medicare Part D and Medicaid managed care programs. The proposed amendment would remove protection for "discounts" from Anti-Kickback enforcement action, and would include criminal and civil penalties for knowingly and willfully offering, paying, soliciting, or receiving remuneration to induce or reward the referral of business reimbursable under federal health care programs. At the same time, HHS also proposed to create a new safe harbor to protect point-of-sale discounts that drug manufacturers provide directly to patients, and adds another safe harbor to protect certain administrative fees paid by manufacturers to PBMs. If this proposal is adopted, in whole or in part, it could affect the pricing and reimbursement for any products for which we receive approval in the future.

The federal false claims and civil monetary penalty laws, including the FCA, which imposes significant penalties and can be enforced by private citizens through civil qui tam actions, prohibit any person or entity from, among other things, knowingly presenting, or causing to be presented, a false or fraudulent claim for payment to, or approval by, the federal healthcare programs, including Medicare and Medicaid, or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. A claim includes "any request or demand" for money or property presented to the U.S. government. Historically, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved, off-label, and thus generally non-reimbursable, uses.

HIPAA created additional federal criminal statutes that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third-party payors, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, 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 Anti-Kickback Statute, the ACA amended the intent standard for certain healthcare fraud statutes under HIPAA such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

Also, many states have similar, and typically more prohibitive, fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor. Additionally, to the extent that our product candidates may in the future be sold in a foreign country, we may be subject to similar foreign laws.

We may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health

Act (“HITECH”) and its implementing regulations, imposes requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA’s privacy and security standards directly applicable to business associates, independent contractors, or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys’ fees and costs associated with pursuing federal civil actions. In addition, many state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways, are often not preempted by HIPAA, and may have a more prohibitive effect than HIPAA, thus complicating compliance efforts. Additionally, more general personal privacy laws have also been enacted by various States, such as Massachusetts, by other countries where we do business, such as member countries of the European Union, that require adoption of policies and procedures to protect, properly store, and to obtain permission to use in our business and clinical research. Failure to comply could result in penalties and interruption of our business should a violation occur.

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We expect our product candidates, once approved, may be eligible for coverage under Medicare, the federal health care program that provides health care benefits to the aged and disabled, and covers outpatient services and supplies, including certain pharmaceutical products, that are medically necessary to treat a beneficiary's health condition. In addition, our product candidates may be covered and reimbursed under other government programs, such as Medicaid and the 340B Drug Pricing Program. The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. Under the 340B Drug Pricing Program, the manufacturer must extend discounts to entities that participate in the program. As part of the requirements to participate in certain government programs, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average manufacturer price, or AMP, and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely.

Additionally, the federal Physician Payments Sunshine Act (the "Sunshine Act"), within the ACA, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) report annually to CMS information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, the physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members. Failure to report accurately could result in penalties. In addition, many states also govern the reporting of payments or other transfers of value, many of which differ from each other in significant ways, are often not preempted, and may have a more prohibitive effect than the Sunshine Act, thus further complicating compliance efforts.

Environment

Our third-party manufacturers are subject to inspections by the FDA for compliance with cGMP and other U.S. regulatory requirements, including U.S. federal, state, and local regulations regarding environmental protection and hazardous and controlled substance controls, among others. Environmental laws and regulations are complex, change frequently, and have tended to become more stringent over time. We have incurred, and may continue to incur, significant expenditures to ensure we are in compliance with these laws and regulations. We would be subject to significant penalties for failure to comply with these laws and regulations.

Employees

As of December 31, 2018, we had 78 full-time employees, of whom 49 are engaged in research and development and 29 in administration. None of our employees are represented by a labor union or covered by a collective bargaining agreement. Geographically, 66 of our employees are located in Massachusetts, nine in Colorado, one in New Jersey, one in New York, and one in North Carolina.

Corporate Information

We were incorporated in Delaware in 2006. We maintain our executive offices at 87 Cambridgepark Drive, Cambridge, MA 02140, and our main telephone number is (617) 621-8097. Our website is located at www.dicerna.com, which contains information about us.

The information in, or that can be accessed through, our website is not part of this Annual Report on Form 10-K. Our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act are available, free of charge, on or through our website as soon as reasonably practicable after such reports and amendments are electronically filed with or furnished to the SEC. The SEC maintains an Internet site that contains reports, proxy and information statements and other information regarding our filings at www.sec.gov.

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 on January 30, 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 exceeds $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 herein as the "JOBS Act," and references herein to "emerging growth company" shall have the meaning associated with it in the JOBS Act.

ITEM 1A. RISK FACTORS

We are providing the following cautionary discussion of risk factors, uncertainties and assumptions that we believe are relevant to our business. These are factors that we believe, individually or in the aggregate, could cause our actual results to differ materially from expected and historical results and our forward-looking statements. We note these factors for investors as permitted by Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended. You should understand that it is not possible to predict or identify all such factors. Consequently, you should not consider this section to be a complete discussion of all potential risks or uncertainties that may substantially impact our business. Moreover, we operate in a competitive and rapidly changing environment. New factors emerge from time to time, and it is not possible to predict the impact of all these factors on our business, financial condition, or results of operations.

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Risks Related to Our Business

We will need to raise substantial additional funds to advance development of our product candidates and we cannot guarantee that we will have sufficient funds available in the future to develop and commercialize our current or future product candidates. Raising additional funds may cause dilution to our stockholders, restrict our operations, or require us to relinquish control over our technologies or product candidates.

We will need to raise substantial additional funds to expand our development, regulatory, manufacturing, marketing, and sales capabilities, whether internally or through other organizations. We have used substantial funds to develop our product candidates and delivery technologies and will require significant funds to conduct further research and development and preclinical testing and clinical trials of our product candidates, to seek regulatory approvals for our product candidates, and to manufacture and market products, if any are approved for commercial sale. As of December 31, 2018, we had $302.6 million in cash, cash equivalents, and held-to-maturity investments. Subsequently, we received an upfront payment in the amount of $5.0 million from Boehringer Ingelheim ("BI"), in connection with BI's exercise of its initial option under the Company's collaboration with BI, an upfront payment in the amount of $100.0 million from the Company's recent collaboration with Eli Lilly and Company ("Lilly"), also made the Company's final payment to Alnylam Pharmaceuticals, Inc. ("Alnylam") in the amount of $10.5 million pursuant to a Confidential Settlement Agreement and General Release. Based on our current operating plan and liquidity, the Company believes that our available cash, cash equivalents, and held-to-maturity investments will be sufficient to fund the execution of our current clinical and operating plan beyond 2020. However, to the extent our clinical and operating plan changes, and to fund our operations beyond 2020, we will need to raise substantial additional funds. Further, our future capital requirements and the period for which our existing resources are able to support our operations may vary significantly from what we expect. Our monthly spending levels vary based on new and ongoing development and corporate activities. Because the length of time and activities associated with successful development of our product candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. To execute our business plan, we will need, among other things:

- to obtain the human and financial resources necessary to develop, test, obtain regulatory approval for, manufacture, and market our product candidates;
- to build and maintain a strong intellectual property portfolio and avoid infringing intellectual property of third parties;
- to establish and maintain successful licenses, collaborations, and alliances;
- to satisfy the requirements of clinical trial protocols, including patient enrollment;
- to establish and demonstrate the clinical efficacy and safety of our product candidates;
- to manage our spending as costs and expenses increase due to preclinical studies and clinical trials, regulatory approvals, manufacturing scale-up, and commercialization;
- to obtain additional capital to support and expand our operations;
- to satisfy the requirements for quality and safety in developing and commercializing our products; and
- to market our products to achieve acceptance and use by the medical community.

If we are unable to obtain funding on a timely basis or on acceptable terms, we may have to delay, reduce, or terminate our research and development programs and preclinical studies or clinical trials, if any, limit strategic opportunities, or undergo reductions in our workforce or other corporate restructuring activities. We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to some of our technologies or product candidates that we would otherwise pursue on our own. We do not expect to realize revenue from product sales or royalties in the foreseeable future, if at all, and milestone payments, if any, are based on third-party determinations and/or events outside our control. Our revenue sources currently are, and will remain, extremely limited unless and until our product candidates are clinically tested, approved for commercialization, and successfully marketed. To date, we have financed our operations primarily through the sale of securities, research collaborations and license agreements, debt financings, and credit and loan facilities. We will be required to seek additional funding in the future and intend to do so through a combination of public or private equity offerings, debt financings, and research collaborations and license agreements. Our ability to raise additional funds will depend on financial, economic, and other factors, many of which are beyond our control. For example, a number of factors, including the timing and outcomes of our clinical activities, our status as a smaller reporting company

under SEC regulations, as well as conditions in the global financial markets, may present significant challenges to accessing the capital markets at a time when we would like or require, and at an increased cost of capital. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity securities, our stockholders will suffer dilution, and the terms of any financing may adversely affect the rights of our stockholders. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Debt financing, if available, may involve restrictive covenants limiting our flexibility in conducting future business activities, and, in the event of insolvency, debt holders would be repaid before holders of equity securities receive any distribution of corporate assets.

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We have a history of operating losses; we expect to continue to incur significant losses for the foreseeable future and may never achieve or maintain profitability, which could result in a decline in the market value of our common stock.

We are a biopharmaceutical company with a limited operating history focused on the discovery and development of treatments based on the emerging therapeutic modality RNAi, a biological process in which RNA molecules inhibit gene expression. Since our inception in October 2006, we have devoted our resources to the development of RNAi molecules and delivery technologies. We have had significant operating losses since our inception. As of December 31, 2018, we had an accumulated deficit of $404.8 million. For the years ended December 31, 2018, 2017, and 2016, our net loss attributable to common stockholders was $88.9 million, $80.3 million, and $59.5 million, respectively. Substantially all of our operating losses have resulted from expenses incurred in connection with our research and development programs, from general and administrative costs associated with our operations, and litigation expenses associated with the Alnylam litigation settled in April 2018. Our technologies and product candidates are in early stages of development, and we are subject to the risks of failure inherent in the development of product candidates based on novel technologies.

We have not generated, and do not expect to generate, any revenue from product sales for the foreseeable future, and we expect to continue to incur significant operating losses for the foreseeable future due to the cost of research and development, preclinical studies and clinical trials, and the regulatory approval process for product candidates. The amount of future losses is uncertain. Our ability to achieve profitability, if ever, will depend on, among other things, us or our existing collaborators, or any future collaborators, successfully developing product candidates, obtaining regulatory approvals to market and commercialize product candidates, manufacturing any approved products on commercially reasonable terms, establishing a sales and marketing organization or suitable third-party alternatives for any approved product, and raising sufficient funds to finance business activities. If we or our existing collaborators, or any future collaborators, are unable to develop and commercialize one or more of our product candidates or if sales revenue from any product candidate that receives approval is insufficient, we will not achieve profitability, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expense related to our product candidates or future development programs;
- delays in initiating or conducting, or release of results of clinical trials, or the addition or termination of clinical trials or funding support by us, our existing collaborators, or any future collaborator or licensor;
- the timing of the release of results from any clinical trials conducted by us or our collaborator BI;
- our execution of any collaboration, licensing, or similar arrangement, and the timing of payments we may make or receive under such existing or future arrangements or the termination or modification of any such existing or future arrangements;
- any intellectual property infringement or misappropriation lawsuit or opposition, interference, re-examination, post-grant review, inter partes review, nullification, derivation action, or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us and our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments, or changes in business strategy;
- if any of our product candidates receive regulatory approval, market acceptance and demand for such product candidates;
- if any of our third-party manufacturers fail to execute on our manufacturing requirements or perform in accordance with cGMP;
- regulatory developments affecting our product candidates or those of our competitors;
- disputes concerning patents, proprietary rights, or license and collaboration agreements that negatively impact our receipt of milestone payments or royalties or require us to make significant payments arising from licenses, settlements, adverse judgments, or ongoing royalties;

- changes in general market and economic conditions; and
- changes in tax laws.

If our quarterly operating results fluctuate or fall below the expectations of investors or securities analysts, the price of our common stock could fluctuate or decline substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

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Our approach to the discovery and development of innovative therapeutic treatments based on novel technologies is unproven and may not result in marketable products.

We plan to develop subcutaneously delivered RNAi-based pharmaceuticals using our GalXC RNAi platform for the treatment of rare diseases involving the liver and for other therapeutic areas involving the liver such as chronic liver diseases, as well as cardiovascular diseases and viral infectious diseases. We believe that product candidates identified with our drug discovery and delivery platform may offer an improved therapeutic approach to small molecules and monoclonal antibodies, as well as several advantages over earlier generation RNAi molecules. However, the scientific research that forms the basis of our efforts to develop product candidates is relatively new. The scientific evidence to support the feasibility of developing therapeutic treatments based on RNAi and GalXC is both preliminary and limited.

Relatively few product candidates based on RNAi have been tested in animals or humans, and a number of clinical trials conducted by other companies using RNAi technologies have not been successful. We may discover that GalXC does not possess certain properties required for a drug to be safe and effective, such as the ability to remain stable in the human body for the period of time required for the drug to reach the target tissue or the ability to cross the cell wall and enter into cells within the target tissue for effective delivery. We currently have only limited data, and no conclusive evidence, to suggest that we can introduce these necessary drug-like properties into GalXC. We may spend substantial funds attempting to introduce these properties and may never succeed in doing so. In addition, product candidates based on GalXC may demonstrate different chemical and pharmacological properties in patients than they do in laboratory studies. Even if product candidates, such as DCR PHXC, have successful results in animal studies, they may not demonstrate the same chemical and pharmacological properties in humans and may interact with human biological systems in unforeseen, ineffective, or harmful ways. As a result, we may never succeed in developing a marketable product, we may not become profitable, and the value of our common stock will decline.

Further, the United States ("U.S.") Food and Drug Administration ("FDA") has relatively limited experience with RNAi or GalXC-based therapeutics. We and our current collaborators, or any future collaborators, may never receive approval to market and commercialize any product candidate. Even if we or a collaborator obtain regulatory approval, the approval may be for disease indications or patient populations that are not as broad as we intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We or a collaborator may be required to perform additional or unanticipated clinical trials to obtain approval or be subject to post-marketing testing requirements to maintain regulatory approval. If our technologies based on GalXC prove to be ineffective, unsafe, or commercially unviable, our entire platform and pipeline would have little, if any, value, which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

The market may not be receptive to our product candidates based on a novel therapeutic modality, and we may not generate any future revenue from the sale or licensing of product candidates.

Even if approval is obtained for a product candidate, we may not generate or sustain revenue from sales of the product due to numerous factors, including whether the product can be sold at a competitive price and otherwise is accepted in the market. The product candidates that we are developing are based on new technologies and therapeutic approaches. Market participants with significant influence over acceptance of new treatments, such as physicians and third-party payors, may not adopt a treatment based on GalXC technology, and we may not be able to convince the medical community and third-party payors, including health insurers, to accept and use, or to provide favorable coverage or reimbursement for, any product candidates developed by us or our existing collaborator or any future collaborators. Market acceptance of our product candidates will depend on, among other factors:

- the timing of our receipt of any marketing and commercialization approvals and those of our competitors;
- the terms of any approvals and the countries in which approvals are obtained;
- the safety and efficacy of our product candidates;
- the prevalence and severity of any adverse side effects associated with our product candidates;
- limitations or warnings contained in any labeling approved by the FDA or other regulatory authority;
- relative convenience and ease of administration of our product candidates;
- the willingness of physicians and patients to accept any new methods of administration;
- the success of our physician education programs;

- the availability of adequate government and third-party payor coverage and reimbursement;
- the pricing of our products, particularly as compared to alternative treatments;
- our ability to compliantly market and sell our products; and

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- availability of alternative effective treatments for the disease indications our product candidates are intended to treat and the relative risks, benefits and costs of those treatments.

With our focus on the emerging therapeutic modality RNAi, these risks may increase to the extent the market becomes more competitive or less favorable to this approach. Additional risks apply to any disease indications we pursue which are for rare diseases. Because of the small patient population for a rare disease, if pricing is not approved or accepted in the market at an appropriate level for an approved rare disease product, such drug may not generate enough revenue to offset costs of development, manufacturing, marketing, and commercialization, despite any benefits received from our efforts to obtain orphan drug designation by regulatory agencies in major commercial markets, such as the U.S., the European Union ("EU"), and Japan. These benefits may include market exclusivity, assistance in clinical trial design, or a reduction in user fees or tax credits related to development expense. Market size is also a variable in disease indications that are not classified as rare. Our estimates regarding potential market size for any indication may be materially different from what we discover to exist if we ever get to the point of product commercialization, which could result in significant changes in our business plan and have a material adverse effect on our business, financial condition, results of operations, and prospects.

If a product candidate that has orphan drug designation subsequently receives the first FDA approval for the indication for that designation, the product candidate is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same product candidate for the same indication, except in very limited circumstances, for seven years. Orphan drug exclusivity, however, could also block the approval of one of our product candidates for seven years if a competitor obtains approval of the same product candidate as defined by the FDA.

Even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective, makes a major contribution to patient care, or meets certain other criteria.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017 ("FDARA"). FDARA, among other things, codified the FDA's preexisting regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The law reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

As in the U.S., we may apply for designation of a product candidate as an orphan drug for the treatment of a specific indication in the EU before the application for marketing authorization is made. For example, in August 2018, the European Medicines Agency's Committee for Orphan Medicinal Products designated DCR PHXC as an orphan medicinal product for the treatment of PH in the EU. Sponsors of orphan drugs in the EU can enjoy economic and marketing benefits, including up to 10 years of market exclusivity for the approved indication. During such period, marketing authorization applications for a "similar medicinal product" will not be accepted, unless another applicant can show that its product is safer, more effective, or otherwise clinically superior to the orphan-designated product. In the EU, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. The respective orphan designation and exclusivity frameworks in the U.S. and in the EU are subject to change, and any such changes may affect our ability to obtain U.S. or EU orphan designations in the future.

Our product candidates are in early stages of development and may fail or suffer delays that materially and adversely affect their commercial viability.

We currently have no products on the market and all of our product candidates are in early stages of development. Our ability to achieve and sustain profitability depends on obtaining regulatory approvals, including ethics committee

approval to conduct clinical trials at particular sites, and successfully commercializing our product candidates, either alone or with third parties, such as our collaborators. Before obtaining regulatory approval for the commercial distribution of our product candidates, we or a collaborator must conduct extensive preclinical and other non-clinical tests and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Non-clinical testing and clinical trials are expensive, difficult to design and implement, can take many years to complete, and are uncertain as to outcome. The start or end of a clinical study is often delayed or halted due to changing regulatory requirements, manufacturing challenges, required clinical trial administrative actions, slower than anticipated patient enrollment, changing standards of care, availability or prevalence of use of a comparative drug or required prior therapy, clinical outcomes, and financial constraints. For instance, delays or difficulties in patient enrollment or difficulties in retaining trial participants can result in increased costs, longer development times, or termination of a clinical trial. Clinical trials of a new product candidate require the

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enrollment of a sufficient number of patients, including patients who are suffering from the disease the product candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, including the size of the patient population, the eligibility criteria for the clinical trial, the age and condition of the patients, the stage and severity of disease, the nature of the protocol, the proximity of patients to clinical sites, and the availability of effective treatments for the relevant disease.

A product candidate can unexpectedly fail at any stage of preclinical and clinical development. The historical failure rate for product candidates is high due to many factors, including scientific feasibility, safety, efficacy, and changing standards of medical care. The results from preclinical testing or early clinical trials of a product candidate may not predict the results that will be obtained in later phase clinical trials of the product candidate. We, the FDA or other applicable regulatory authorities, an individual Institutional Review Board ("IRB") with respect to its institution, or an independent ethics committee may suspend clinical trials of a product candidate at any time for various reasons, including a belief that individuals participating in such trials are being exposed to unacceptable health risks or adverse side effects. We may not have the financial resources to continue development of, or to enter into collaborations for, a product candidate if we experience any problems or other unforeseen events that delay or prevent regulatory approval of, or our ability to commercialize, product candidates, including:

- negative or inconclusive results from our clinical trials or the clinical trials of others for product candidates similar to ours, leading to a decision or requirement to conduct additional preclinical testing or clinical trials or abandon a program;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- delays in submitting INDs or comparable foreign applications or delays or failure in obtaining the necessary approvals from regulators or IRBs to commence a clinical trial, or a suspension or termination of a clinical trial once commenced;
- conditions imposed by the FDA or comparable foreign authorities, such as the European Medicines Agency ("EMA"), regarding the scope or design of our clinical trials;
- delays in enrolling individuals in clinical trials;
- high drop-out rates of study participants;
- inadequate supply or quality of drug product or product candidate components or materials or other supplies necessary for the conduct of our clinical trials;
- greater than anticipated clinical trial costs;
- poor effectiveness of our product candidates during clinical trials;
- unfavorable FDA or other regulatory agency inspection and review of a clinical trial site;
- failure of our third-party contractors or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner, or at all;
- delays and changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around clinical testing generally or with respect to our technology in particular; and
- varying interpretations of data by the FDA and foreign regulatory agencies.

We are dependent on our collaboration partners for the successful development of product candidates and, therefore, are subject to the efforts of these partners and our ability to successfully collaborate with these partners.

We have entered into collaboration agreements with Lilly, an affiliate of Alexion Pharmaceuticals, Inc. (together with its affiliates, "Alexion"), and BI (our "Collaboration Partners") providing joint development of certain RNAi therapies. The success of our collaborations with our Collaboration Partners and the realization of the milestone and royalty payments under the collaboration agreements depends upon the efforts of our Collaboration Partners, any of which may not be successful in obtaining approvals for the product candidates developed under the collaboration or in marketing, or arranging for necessary supply, manufacturing, or distribution relationships for, any approved products. Our Collaboration Partners may change their strategic focus or pursue alternative technologies in a manner that results in reduced, delayed, or no additional payments to us under the collaboration agreements. Our Collaboration Partners have a variety of marketed products and product candidates under collaboration with other companies, possibly including some of our competitors, and BI's own corporate objectives may not be consistent with our interests. If our

Collaboration Partners fail to develop, obtain regulatory approval for, or ultimately commercialize any product candidate under our collaborations, or if any of our Collaboration Partners terminates their applicable collaboration, our business, financial condition, results of operations, and prospects could be materially and adversely affected. Each of our collaboration agreements is terminable by the applicable collaboration partner any time at will, subject to compliance with applicable notice periods. In addition, if we have a dispute or enter into litigation with any of our Collaboration Partners in the future, it could delay development programs, create uncertainty as to ownership of intellectual property rights, distract management from other business activities, and generate substantial expense.

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If third parties on which we depend to conduct our preclinical studies, or any future clinical trials, do not perform as contractually required, fail to satisfy regulatory or legal requirements, or miss expected deadlines, our development program could be delayed with materially adverse effects on our business, financial condition, results of operations, and prospects.

We rely on third-party clinical investigators, contract research organizations ("CROs"), clinical data management organizations, and consultants to design, conduct, supervise, and monitor preclinical studies of our product candidates and will do the same for any clinical trials. Because we rely on third parties and do not have the ability to conduct preclinical studies or clinical trials independently, we have less control over the timing, quality, compliance, and other aspects of preclinical studies and clinical trials than we would if we conducted them on our own. These investigators, CROs, and consultants are not our employees and we have limited control over the amount of time and resources that they dedicate to our programs. These third parties may have contractual relationships with other entities, some of which may be our competitors, which may draw time and resources from our programs. The third parties with which we contract might not be diligent, careful, compliant, or timely in conducting our preclinical studies or clinical trials, resulting in the preclinical studies or clinical trials being delayed or unsuccessful.

If we cannot contract with acceptable third parties on commercially reasonable terms, or at all, or if these third parties do not carry out their contractual duties, satisfy legal and regulatory requirements for the conduct of preclinical studies or clinical trials, or meet expected deadlines, our clinical development programs could be delayed and otherwise adversely affected. In all events, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the general investigational plan and protocols for the trial as well as applicable laws and regulations. The FDA and certain foreign regulatory authorities, such as the EMA, require preclinical studies to be conducted in accordance with applicable good laboratory practices and clinical trials to be conducted in accordance with applicable FDA regulations and applicable good clinical practices, including requirements for conducting, recording, and reporting the results of preclinical studies and clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity, and confidentiality of clinical trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Any such event could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Because we rely on third-party manufacturing and supply partners, our supply of research and development, preclinical studies, and clinical trial materials may become limited or interrupted or may not be of satisfactory quantity or quality.

We rely on third-party supply and manufacturing companies and organizations to supply the materials, components, and manufacturing services for our research and development, preclinical study, and clinical trial drug supplies. We do not own or lease manufacturing facilities or supply sources for such components and materials. Our manufacturing requirements include oligonucleotides and custom amidites, some of which we procure from a single source supplier on a purchase order basis. In addition, for each product candidate, we typically contract with only one manufacturer for the formulation and filling of drug product. There can be no assurance that our supply of research and development, preclinical study, and clinical trial drugs and other materials will not be limited, interrupted, restricted in certain geographic regions, or of satisfactory quality, or continue to be available at acceptable prices. In particular, any replacement of our drug substance manufacturer could require significant effort and expertise because there may be a limited number of qualified replacements.

If we are at any time unable to provide an uninterrupted supply of our product candidates or, following regulatory approval, any products to patients, we may lose patients, physicians may elect to utilize competing therapeutics instead of our products, and our clinical trials may be adversely affected, which could materially and adversely affect our clinical trial outcomes.

The manufacturing process for a product candidate is subject to FDA and foreign regulatory authority review. Suppliers and manufacturers must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory standards, such as current good manufacturing practices ("cGMP"). In the event that any of our suppliers or manufacturers fails to comply with such requirements or to perform its obligations regarding quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may experience shortages resulting in delayed

shipments, supply constraints and/or stock-outs of our products, be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture our product candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills or technology to another third party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third party manufacture our product candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates in a timely manner or within budget.

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We expect to continue to rely on third-party manufacturers if we receive regulatory approval for any product candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third-party manufacturing for product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Our or a third party's failure to execute on our manufacturing requirements could adversely affect our business in a number of ways, including:

- an inability to initiate or continue preclinical studies or clinical trials of product candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for product candidates;
- lack of or loss of the cooperation of a collaborator;
- subjecting manufacturing facilities of our product candidates to additional inspections by regulatory authorities;
- requirements to cease distribution or to recall batches of our product candidates; and
- in the event of approval to market and commercialize a product candidate, an inability to meet commercial demands for our products.

We may not successfully engage in strategic transactions, including any additional collaborations we seek, which could adversely affect our ability to develop and commercialize product candidates, impact our cash position, increase our expense, and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as collaborations, acquisitions of companies, asset purchases, and out- or in-licensing of product candidates or technologies. In addition to our current collaborations with BI, Alexion, and Lilly, we will evaluate and, if strategically attractive, seek to enter into additional collaborations, including with major biopharmaceutical, biotechnology, or pharmaceutical companies. The competition for collaborators is intense, and the negotiation process is time-consuming and complex. Any new collaboration may be on terms that are not optimal for us, and we may be unable to maintain any new or existing collaboration if, for example, development or approval of a product candidate is delayed, sales of an approved product do not meet expectations, or the collaborator terminates the collaboration. Any such collaboration, or other strategic transaction, may require us to incur non-recurring or other charges, increase our near- and long-term expenditures, and pose significant integration or implementation challenges or disrupt our management or business. These transactions entail numerous operational and financial risks, including exposure to unknown liabilities, disruption of our business, and diversion of our management's time and attention in order to obtain and manage a collaboration or develop acquired products, product candidates, or technologies, incurrence of substantial debt or dilutive issuances of equity securities to pay transaction consideration or costs, higher than expected collaboration, acquisition, or integration costs, write-downs of assets or goodwill or impairment charges, increased amortization expenses, difficulty and cost in facilitating the collaboration or combining the operations and personnel of any acquired business, deterioration of relationships with key suppliers, manufacturers, or customers of any acquired business due to changes in management and ownership and the inability to retain key employees of any acquired business. Accordingly, although there can be no assurance that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks and have a material adverse effect on our business, results of operations, financial condition, and prospects. Conversely, failure to enter any collaboration or other strategic transaction that would be beneficial to us could delay the development and potential commercialization of our product candidates and have a negative impact on the competitiveness of any product candidate that reaches market.

We face competition from entities that have developed or may develop product candidates for our target disease indications, including companies developing novel treatments and technology platforms based on modalities and technology similar to ours. If these companies develop technologies or product candidates more rapidly than we do or their technologies, including delivery technologies, are more effective, our ability to develop and successfully commercialize product candidates may be adversely affected.

The development and commercialization of drugs is highly competitive. We compete with a variety of multinational pharmaceutical companies and specialized biotechnology companies, as well as technology being developed at universities and other research institutions. Our competitors have developed, are developing, or may develop product

candidates and processes competitive with our product candidates, some of which may become commercially available before any of our product candidates. Competitive therapeutic treatments include those that have already been approved and accepted by the medical community and any new treatments that enter the market. We are aware of many companies that are working in the field of RNAi therapeutics, including major pharmaceutical companies and a number of biopharmaceutical companies including Alnylam, Arrowhead Pharmaceuticals, Inc. (“Arrowhead”), and Arbutus Biopharma Corporation. We believe that a significant number of products are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may try to develop product candidates.

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We also compete with companies working to develop antisense and other RNA-based drugs. Like RNAi therapeutics, antisense drugs target mRNA with the objective of suppressing the activity of specific genes. The development of antisense drugs is more advanced than that of RNAi therapeutics, and antisense technology may become the preferred technology for products that target mRNAs. Significant competition also exists from companies such as Alnylam and Arrowhead to discover and develop safe and effective means to deliver therapeutic RNAi molecules to the relevant cell and tissue types.

Many of our competitors have significantly greater financial, technical, manufacturing, marketing, sales and supply resources or experience than we have. If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including safety and effectiveness, ease with which our products can be administered and the extent to which patients and physicians accept relatively new routes of administration, timing and scope of regulatory approvals, availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage, and patent position of our products. Competing products could present superior treatment alternatives, including by being more effective, safer, less expensive or marketed and sold more effectively than any products we may develop. Competitive products may make any products we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. Competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan.

Any inability to attract and retain qualified key management and technical personnel would impair our ability to implement our business plan.

Our success largely depends on the continued service of key management and other specialized personnel, including: Douglas M. Fambrough, III, Ph.D., our chief executive officer; Bob D. Brown, Ph.D., our chief scientific officer; Ralf Rosskamp, M.D., our chief medical officer; John B. Green, our chief financial officer; and James B. Weissman, our chief business officer. The loss of one or more members of our management team or other key employees or advisors could delay our research and development programs and materially harm our business, financial condition, results of operations, and prospects. The relationships that our key managers have cultivated within our industry make us particularly dependent upon their continued employment with us. We are dependent on the continued service of our technical personnel because of the highly complex nature of our product candidates and technologies and the specialized nature of the regulatory approval process. Because our management team and key employees are not obligated to provide us with continued service, they could terminate their employment with us at any time without penalty. We do not maintain key person life insurance policies on any of our management team members or key employees. Our future success will depend in large part on our continued ability to attract and retain other highly qualified scientific, technical, and management personnel, as well as personnel with expertise in clinical testing, manufacturing, governmental regulation and commercialization. We face competition for personnel from other companies, universities, public and private research institutions, government entities, and other organizations.

If our product candidates advance into clinical trials, we may experience difficulties in managing our growth and expanding our operations.

We have limited experience in drug development and very limited experience with clinical trials of product candidates. As our product candidates enter and advance through preclinical studies and any clinical trials, we will need to expand our development, regulatory, and manufacturing capabilities or contract with other organizations to provide these capabilities for us. In the future, we expect to have to manage additional relationships with collaborators, suppliers, and other organizations. Our ability to manage our operations and future growth will require us to continue to improve our operational, financial, and management controls, reporting systems, and procedures. We may not be able to implement improvements to our management information and control systems in an efficient or timely manner and may discover deficiencies in existing systems and controls.

If any of our product candidates are approved for marketing and commercialization and we are unable to develop sales, marketing, and distribution capabilities on our own or enter into agreements with third parties to perform these functions on acceptable terms, we will be unable to successfully commercialize any such future products.

We currently have no sales, marketing, or distribution capabilities or experience. If any of our product candidates are approved, we will need to develop internal sales, marketing, and distribution capabilities to commercialize such

products, which would be expensive and time-consuming, or enter into collaborations with third parties to perform these services. If we decide to market our products directly, we will need to commit significant financial, legal, and managerial resources to develop a marketing and sales force with technical expertise and supporting distribution, administration, and compliance capabilities. If we rely on third parties with such capabilities to market our approved products or decide to co-promote products with collaborators, we will need to establish and maintain marketing and distribution arrangements with third parties, and there can be no assurance that we will be able to enter into such arrangements on acceptable, compliant terms, or at all. In entering into third-party marketing or distribution arrangements, any revenue we receive will depend upon the efforts of the third parties and there can be no assurance that such third parties will establish adequate sales and distribution capabilities or be successful in gaining market acceptance of any approved product. If we are not

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successful in commercializing any product approved in the future, either on our own or through third parties, our business, financial condition, results of operations, and prospects could be materially and adversely affected.

If we fail to comply with U.S. and foreign regulatory requirements, regulatory authorities could limit or withdraw any marketing or commercialization approvals we may receive and subject us to other penalties that could materially harm our business.

The Company, our product candidates, our suppliers, and our contract manufacturers, distributors, and contract testing laboratories are subject to extensive regulation by governmental authorities in the EU, the U.S., and other countries, with the regulations differing from country to country.

Even if we receive marketing and commercialization approval of a product candidate, we and our third-party services providers will be subject to continuing regulatory requirements, including a broad array of regulations related to establishment registration and product listing, manufacturing processes, risk management measures, quality and pharmacovigilance systems, post-approval clinical studies, labeling, advertising and promotional activities, record keeping, distribution, adverse event reporting, import and export of pharmaceutical products, pricing, sales and marketing, and fraud and abuse requirements. We are required to submit safety and other post market information and reports and are subject to continuing regulatory review, including in relation to adverse patient experiences with the product and clinical results that are reported after a product is made commercially available, both in the U.S. and any foreign jurisdiction in which we seek regulatory approval. The FDA and certain foreign regulatory authorities, such as the EMA, have significant post-market authority, including the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials to evaluate safety risks related to the use of a product or to require withdrawal of the product from the market. The FDA also has the authority to require a risk evaluation and mitigation strategy ("REMS") plan after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug. The EMA now routinely requires risk management plans ("RMPs") as part of the marketing authorization application process, and such plans must be continually modified and updated throughout the lifetime of the product as new information becomes available. In addition, for nationally authorized medicinal products, the relevant governmental authority of any EU member state can request an RMP whenever there is a concern about a risk affecting the benefit risk balance of the product. The manufacturer and manufacturing facilities we use to make a future product, if any, will also be subject to periodic review and inspection by the FDA and other regulatory agencies, including for continued compliance with cGMP requirements. The discovery of any new or previously unknown problems with our third-party manufacturers, manufacturing processes, or facilities may result in restrictions on the product, manufacturer, or facility, including withdrawal of the product from the market. If we rely on third-party manufacturers, we will not have control over compliance with applicable rules and regulations by such manufacturers. Any product promotion and advertising will also be subject to regulatory requirements and continuing regulatory review. If we or our collaborators, manufacturers, or service providers fail to comply with applicable continuing regulatory requirements in the U.S. or foreign jurisdictions in which we seek to market our products, we or they may be subject to, among other things, fines, warning and untitled letters, clinical holds, delay or refusal by the FDA or foreign regulatory authorities to approve pending applications or supplements to approved applications, suspension, refusal to renew or withdrawal of regulatory approval, product recalls, seizures or administrative detention of products, refusal to permit the import or export of products, operating restrictions, inability to participate in government programs including Medicare and Medicaid, and total or partial suspension of production or distribution, injunction, restitution, disgorgement, debarment, civil penalties, and criminal prosecution.

We face risks arising from the results of the public referendum held in United Kingdom and its membership in the European Union.

We have a subsidiary located in the United Kingdom (the "UK"), which we established in order to allow us to conduct clinical trials in EU member states. On June 23, 2016, the UK held a referendum in which voters approved an exit from the EU, commonly referred to as "Brexit." The withdrawal of the UK from the EU will take effect either on the effective date of the withdrawal agreement or, in the absence of agreement, two years after the UK provides a notice of withdrawal pursuant to the EU Treaty. On March 29, 2017, the Prime Minister of the UK delivered a formal notice of withdrawal to the EU. On May 22, 2017, the Council of the EU (the "Council"), adopted a decision authorizing

the opening of Brexit negotiations with the UK and formally nominated the European Commission as EU negotiator. The Council also adopted negotiating directives for the talks, which began on April 18, 2018. Because of the regulatory uncertainty surrounding Brexit, we have established a subsidiary in Ireland for ongoing regulatory initiatives in the EU.

The ongoing developments following from the UK's public referendum vote to exit from the EU could cause disruptions to and create uncertainty surrounding our business, including affecting our relationships with existing and potential suppliers, manufacturers, and other third parties. Negotiations have commenced to determine the terms of the UK's future relationship with the EU, including the terms of trade between the UK and the EU. The effects of Brexit will depend upon any agreements the UK makes to retain access to EU markets either during a transitional period or more permanently. The measures could potentially have corporate structural consequences, adversely change tax benefits or liabilities in these or other jurisdictions and could disrupt some of the markets and

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jurisdictions in which we operate. In addition, Brexit could lead to legal uncertainty and potentially divergent national laws and regulations as the UK determines which EU laws to replace or replicate. In addition, the announcement of Brexit has caused significant volatility in global stock markets and currency exchange rate fluctuations, including the strengthening of the USD against some foreign currencies, and the Brexit negotiations may continue to cause significant volatility. The progress and outcomes of Brexit negotiations also may create global economic uncertainty. Any of these effects of Brexit, among others, could materially adversely affect the business, business opportunities, and financial condition of our company.

Price controls imposed in foreign markets and downward pricing pressure in the U.S. may adversely affect our future profitability.

In some countries, particularly member states of the EU, the pricing of prescription drugs may be subject to governmental control, at national as well as at regional levels. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a product. In addition, in the U.S. and elsewhere, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic, and regulatory developments may further complicate pricing and reimbursement negotiations, and pricing negotiations may continue after coverage or reimbursement has been obtained. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we or our collaborators may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our RNAi therapeutic candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of any product candidate approved for marketing is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business, financial condition, results of operations, or prospects could be adversely affected.

Our business entails a significant risk of product liability and our ability to obtain sufficient insurance coverage could harm our business, financial condition, results of operations, or prospects.

Our business exposes us to significant product liability risks inherent in the development, testing, manufacturing, and marketing of therapeutic treatments. Product liability claims could delay or prevent completion of our development programs. If we succeed in marketing products, such claims could result in an investigation by certain regulatory authorities, such as the FDA or foreign regulatory authorities, of the safety and effectiveness of our products, our manufacturing processes and facilities, or our marketing programs and potentially a recall of our products or more serious enforcement action, limitations on the approved indications for which they may be used, or suspension or withdrawal of approvals. Regardless of the merits or eventual outcome, liability claims may also result in decreased demand for our products, injury to our reputation, costs to defend related litigation, a diversion of management's time and our resources, substantial monetary awards to clinical trial participants or patients, and a decline in our stock price. We currently have product liability insurance that we believe is appropriate for our stage of development and may need to obtain higher levels prior to marketing any of our product candidates. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have a material adverse effect on our business.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have a material adverse effect on our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include, but is not limited to, intentional failures to comply with FDA or U.S. healthcare laws and regulations or applicable laws, regulations, guidance, or codes of conduct set by foreign governmental authorities or self-regulatory industry organizations, provide accurate information to any governmental authorities such as the FDA, comply with manufacturing standards we may establish, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing, and business arrangements in the healthcare industry are subject to extensive laws, regulations,

guidance, and codes of conduct intended to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws, regulations, guidance, and codes of conduct may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs, business or conduct involving healthcare professionals, and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions, including debarment or disqualification of those employees from participation in FDA-regulated activities, and serious harm to our reputation. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, regulations, guidance, or codes of conduct. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines, exclusion from government programs, or other sanctions.

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Our internal computer systems, or those of third parties with which we do business, including our CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs or the theft of Company or patient confidential information.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we do business, including our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war, and telecommunication and electrical failures. Such events could cause interruptions of our operations. For instance, the loss of preclinical data or data from any future clinical trial involving our product candidates could result in delays in our development and regulatory filing efforts and significantly increase our costs. Certain data breaches must also be reported to affected individuals and the government, and in some cases to the media, under provisions of the U.S. federal Health Insurance Portability and Accountability Act ("HIPAA"), as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive, and financial penalties may also apply. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data, or inappropriate disclosure of confidential or proprietary information of the Company or patients, we could incur liability and the development of our product candidates could be delayed.

If we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.

Our research, development, and manufacturing involve the use of hazardous materials and various chemicals. We maintain quantities of various flammable and toxic chemicals in our facilities in Cambridge, Massachusetts, that are required for our research, development, and manufacturing activities. We are subject to federal, state, and local laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. We believe our procedures for storing, handling, and disposing these materials in our Cambridge facilities comply with the relevant guidelines of Cambridge, the Commonwealth of Massachusetts, and the Occupational Safety and Health Administration of the U.S. Department of Labor. Although we believe that our safety procedures for handling and disposing of these materials comply with the standards mandated by applicable regulations, the risk of accidental contamination or injury from these materials cannot be eliminated. If an accident occurs, we could be held liable for resulting damages, which could be substantial. We are also subject to numerous environmental, health, and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens, and the handling of animals and biohazardous materials. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of these materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological or hazardous materials. Additional federal, state, and local laws and regulations affecting our operations may be adopted in the future. We may incur substantial costs to comply with, and substantial fines or penalties if we violate any of these laws or regulations.

Our information technology systems could face serious disruptions that could adversely affect our business.

Despite the use of off-site (cloud-based) information storage systems for certain key corporate information, our internal information technology and other infrastructure systems, including corporate firewalls, servers, leased lines, and connection to the Internet, face the risk of systemic failure that could disrupt our operations. A significant disruption in the availability of our information technology and other internal infrastructure systems could cause interruptions in our collaborations and delays in our research and development work.

Our current operations are largely concentrated in one location and any events affecting this location may have material adverse consequences.

Our current operations are carried out primarily in our facilities located in Cambridge, Massachusetts. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure, or other natural or manmade accidents, or incidents that prevent us from fully utilizing the facilities, may have a material adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities

may result in increased costs, delays in the development of our product candidates, or interruption of our business operations. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material adverse effect on our business, financial position, results of operations, and prospects.

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

We have incurred substantial losses during our history, do not expect to become profitable for the foreseeable future, and may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. We may be unable to use these losses to offset income before such unused losses expire. Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50 percentage point change by value in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be further limited. We are in the process of performing an analysis on whether we have experienced any ownership changes in the past. Our preliminary analysis indicates that we may have experienced ownership changes in November 2007, October 2010, February 2014, and December 2017. While this analysis is still preliminary, it is likely that our net operating losses are subject to such limitation. As of December 31, 2018, we had significant U.S. federal and Massachusetts net operating loss carryforwards that could be reduced or lost if we have or do experience an ownership change, which could have an adverse effect on our business, financial position, results of operations, and prospects.

The investment of our cash and cash equivalents and held-to-maturity investments is subject to risks which may cause losses and affect the liquidity of these investments.

As of December 31, 2018, we had $302.6 million in cash and cash equivalents and held-to-maturity investments. We historically have invested substantially all of our available cash and cash equivalents in corporate bonds, commercial paper, securities issued by the U.S. government, certificates of deposit, and money market funds meeting the criteria of our investment policy, which is focused on the preservation of our capital. These investments are subject to general credit, liquidity, market, and interest rate risks. For example, the impact of U.S. sub-prime mortgage defaults in recent years affected various sectors of the financial markets and caused credit and liquidity issues. We may realize losses in the fair value of these investments or a complete loss of these investments, which would have a negative effect on our consolidated financial statements.

In addition, should our investments cease paying or reduce the amount of interest paid to us, our interest income would suffer. The market risks associated with our investment portfolio may have an adverse effect on our results of operations, liquidity, and financial condition.

Changes in accounting rules and regulations, or interpretations thereof, could result in unfavorable accounting charges or require us to change our compensation policies.

Accounting methods and policies for public companies and biopharmaceutical companies, including policies governing revenue recognition, research and development and related expenses, and accounting for stock-based compensation, are subject to review, interpretation, and guidance from our auditors and relevant accounting authorities, including the SEC. Changes to accounting methods or policies, or interpretations thereof, may require us to reclassify, restate, or otherwise change or revise our consolidated financial statements, including those contained in our Annual Reports on Form 10-K.

Risks Related to Intellectual Property

If we are not able to obtain and enforce patent protection for our technologies or product candidates, development and commercialization of our product candidates may be adversely affected.

Our success depends in part on our ability to obtain and maintain patents and other forms of intellectual property rights, including in-licenses of intellectual property rights of others, for our product candidates, methods used to manufacture our product candidates and methods for treating patients using our product candidates, as well as our ability to preserve our trade secrets, to prevent third parties from infringing upon our proprietary rights, and to operate without infringing upon the proprietary rights of others. There can be no assurance that an issued patent will remain valid and enforceable in a court of law through the entire patent term. Should the validity of a patent be challenged, the legal process associated with defending the patent may be costly and time consuming. Issued patents can be subject to oppositions, interferences, post-grant proceedings, and other third-party challenges that can result in the revocation of the patent or limit patent claims such that patent coverage lacks sufficient breadth to protect subject matter that is commercially relevant. Competitors may be able to circumvent our patents. Development and commercialization of pharmaceutical products can be subject to substantial delays and it is possible that at the time of

commercialization any patent covering the product will have expired or will be in force for only a short period of time thereafter.

As of March 4, 2019, our worldwide patent estate, not including the patents and patent applications that we have licensed from third parties, included over 55 issued patents or allowed patent applications and at least 80 pending patent applications supporting commercial development of our RNAi molecules and delivery technologies. We may not be able to apply for patents on certain aspects of our product candidates or delivery technologies in a timely fashion or at all. Our existing issued and granted patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing

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products and technology. There is no guarantee that any of our pending patent applications will result in issued or granted patents, that any of our issued or granted patents will not later be found to be invalid or unenforceable, or that any issued or granted patents will include claims that are sufficiently broad to cover our product candidates or delivery technologies or to provide meaningful protection from our competitors. Moreover, the patent position of biotechnology and pharmaceutical companies can be highly uncertain because it involves complex legal and factual questions. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our current and future proprietary technology and product candidates are covered by valid and enforceable patents or are effectively maintained as trade secrets. If third parties disclose or misappropriate our proprietary rights, it may materially and adversely impact our position in the market.

The U.S. Patent and Trademark Office ("USPTO") and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment, and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case. The standards applied by the USPTO and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. As such, we do not know the degree of future protection that we will have on our proprietary products and technology. While we will endeavor to protect our product candidates with intellectual property rights such as patents, as appropriate, the process of obtaining patents is time-consuming, expensive, and sometimes unpredictable.

In addition, there are numerous recent changes to the patent laws and proposed changes to the rules of the USPTO which may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. The U.S. Supreme Court has ruled on several patent cases in recent years, some of which cases either narrow the scope of patent protection available in certain circumstances or weaken the rights of patent owners in certain situations. The 2013 decision by the U.S. Supreme Court in Association for Molecular Pathology v. Myriad Genetics, Inc. precludes a claim to a nucleic acid having a stated nucleotide sequence which is identical to a sequence found in nature and unmodified. We currently are not aware of an immediate impact of this decision on our patents or patent applications because we are developing nucleic acid products that are not found in nature. However, this decision has yet to be clearly interpreted by courts and by the USPTO. We cannot assure you that the interpretations of this decision or subsequent rulings will not adversely impact our patents or patent applications. 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 U.S. 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.

Once granted, patents may remain open to opposition, interference, re-examination, post-grant review, inter partes review, nullification or derivation action in court or before patent offices or similar proceedings for a given period before or after allowance or grant, during which time third parties can raise objections against such initial grant. In the course of such proceedings, which may continue for a protracted period of time, the patent owner may be compelled to limit the scope of the allowed or granted claims thus attacked or may lose the allowed or granted claims altogether.

Our patent risks include that:

- others may, or may be able to, make, use, or sell compounds that are the same as or similar to our product candidates but that are not covered by the claims of the patents that we own or license;
- we or our licensors, collaborators, or any future collaborators may not be the first to file patent applications covering certain aspects of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- a third party may challenge our patents, and, if challenged, a court may not hold that our patents are valid, enforceable, and infringed;

- a third party may challenge our patents in various patent offices, and, if challenged, we may be compelled to limit the scope of our allowed or granted claims or lose the allowed or granted claims altogether;
- any issued patents that we own or have licensed from others may not provide us with any competitive advantages, or may be challenged by third parties;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others could harm our business; and
- our competitors could conduct research and development activities in countries where we will not have enforceable patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets.

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Intellectual property rights of third parties could adversely affect our ability to commercialize our product candidates, and we might be required to litigate or obtain licenses from third parties in order to develop or market our product candidates. Such litigation could be costly and licenses may be unavailable on commercially reasonable terms.

Research and development of RNAi-based therapeutics and other oligonucleotide-based therapeutics has resulted in many patents and patent applications from organizations and individuals seeking to obtain patent protection in the field. Our efforts are based on RNAi technology that we have licensed and that we have developed internally and own or co-own. We have chosen this approach to increase our likelihood of technical success and our freedom to operate. We have obtained grants and issuances of RNAi-based patents and have licensed other patents from third parties on exclusive and non-exclusive bases. The issued patents and pending patent applications in the U.S. and in key markets around the world that we own, co-own, or license claim many different methods, compositions, and processes relating to the discovery, development, manufacture, and commercialization of RNAi therapeutics. Specifically, we own, co-own, or have licensed a portfolio of patents, patent applications, and other intellectual property covering: (1) certain aspects of the structure and uses of RNAi molecules, including their manufacture and use as therapeutics, and RNAi-related mechanisms, (2) chemical modifications to RNAi molecules that improve their properties and suitability for therapeutic uses, (3) RNAi molecules directed to specific gene sequences and drug targets as treatments for particular diseases, and (4) delivery technologies, such as in the field of lipid nanoparticles and lipid nanoparticle formulation, and chemical modifications such as conjugation to targeting moieties.

The RNAi-related intellectual property landscape, including patent applications in prosecution where no definitive claims have yet issued, is still evolving, and it is difficult to conclusively assess our freedom to operate. Other companies are pursuing patent applications and possess issued patents broadly directed to RNAi compositions, methods of making and using RNAi, and to RNAi-related delivery and modification technologies. Our competitive position may suffer if patents issued to third parties cover our products, or our manufacture or uses relevant to our commercialization plans. In such cases, we may not be in a position to commercialize products unless we enter into a license agreement with the intellectual property right holder, if available, on commercially reasonable terms or successfully pursue litigation, opposition, interference, re-examination, post-grant review, inter partes review, nullification, derivation action, or cancellation proceeding to limit, nullify, or invalidate the third-party intellectual property right concerned. Even if we are successful in limiting, nullifying, or invalidating third-party intellectual property rights through such proceedings, we may incur substantial costs and could require significant time and attention of our personnel.

While we believe our intellectual property allows us to pursue our current development programs, the biological process of RNAi is a natural process and cannot be patented. Several companies in the space are pursuing alternate methods to exploit this phenomenon and have built their intellectual property around these methods. For example, Alnylam controls three patent families containing both pending patent applications and issued patents (e.g., U.S. Patent Numbers 8,853,384 and 9,074,213, and European Patent EP 1 352 061 B1) that pertain to RNAi. These are referred to in their corporate literature as the “Tuschl family” (e.g. patents and applications claiming priority to WO2002/044321, filed November 29, 2001, and their priority filings) and the “Kreutzer-Limmer family” (e.g. patents and applications claiming priority to WO 2002/044895, filed January 29, 2000, WO 2002/055693, filed January 9, 2002, and their priority filings). Both families contain patent applications still in prosecution, with the applicants actively seeking to extend the reach of this intellectual property in ways that might strategically impact our business. Additional areas of intellectual property pursued by Alnylam and others include oligonucleotide delivery-related technologies (such as conjugation to targeting moieties) and oligonucleotides directed to specific gene targets. In addition, Silence Therapeutics owns patents directed to certain chemical modifications of RNAi molecules, including U.S. Patent Number 9,222,092, with a priority date of August 5, 2002.

Patent applications in the U.S. and elsewhere are generally published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our product candidates or platform technology could have been filed by others without our knowledge. Additionally, pending claims in patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our platform technologies, our product candidates, or the use of our product candidates. Third-party intellectual property right holders may also bring patent infringement

claims against us. No such patent infringement actions have been brought against us. We cannot guarantee that we will be able to successfully settle or otherwise resolve any future infringement claims. If we are unable to successfully settle future claims on terms acceptable to us, we may be required to engage in or continue costly, unpredictable, and time-consuming litigation, and may be prevented from or experience substantial delays in marketing our products. If we fail in any such dispute, in addition to being forced to pay damages, we may be temporarily or permanently prohibited from commercializing any of our product candidates that are held to be infringing. We might also be forced to redesign product candidates so that we no longer infringe the third-party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

As the field of RNAi therapeutics matures, patent applications are being processed by national patent offices around the world. There is uncertainty about which patents they will issue, and, if they do, as to when, to whom, and with what claims. It is likely that there will be significant litigation in the courts and other proceedings, such as interference, re-examination, opposition, post-grant review, inter partes review, nullification, derivation action, or cancellation proceedings, in various patent offices relating to patent

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rights in the RNAi therapeutics field. In many cases, the possibility of appeal or opposition exists for either us or our opponents, and it may be years before final, unappealable rulings are made with respect to these patents in certain jurisdictions. The timing and outcome of these and other proceedings is uncertain and may adversely affect our business if we are not successful in defending the patentability and scope of our pending and issued patent claims or if third parties are successful in obtaining claims that cover our RNAi technology or any of our product candidates. In addition, third parties may attempt to invalidate our intellectual property rights. Even if our rights are not directly challenged, disputes could lead to the weakening of our intellectual property rights. Our defense against any attempt by third parties to circumvent or invalidate our intellectual property rights could be costly to us, could require significant time and attention of our management, and could have a material adverse effect on our business and our ability to successfully compete in the field of RNAi therapeutics.

There are many issued and pending patents that claim aspects of oligonucleotide chemistry and modifications that we may need to apply to our therapeutic candidates. There are also many issued patents that claim targeting genes or portions of genes that may be relevant for drugs we wish to develop. Thus, it is possible that one or more organizations will hold patent rights to which we will need a license. If those organizations refuse to grant us a license to such patent rights on reasonable terms, we may be unable to market products or perform research and development or other activities covered by these patents.

We may license patent rights from third-party owners or licensees. If such owners or licensees do not properly or successfully obtain, maintain, or enforce the patents underlying such licenses, or if they retain or license to others any competing rights, our competitive position and business prospects may be adversely affected.

We may, in the future, rely on intellectual property rights licensed from third parties to protect our technology, including licenses that give us rights to third-party intellectual property that is necessary or useful for our business. We also may license additional third-party intellectual property in the future. Our success may depend in part on the ability of our licensors to obtain, maintain, and enforce patent protection for our licensed intellectual property, in particular, those patents to which we have secured exclusive rights. Our licensors may not successfully prosecute the patent applications licensed to us. Even if patents issue or are granted, our licensors may fail to maintain these patents, may determine not to pursue litigation against other companies that are infringing these patents, or may pursue litigation less aggressively than we would. Further, we may not obtain exclusive rights, which would allow for third parties to develop competing products. Without protection for, or exclusive right to, the intellectual property we license, other companies might be able to offer substantially identical products for sale, which could adversely affect our competitive business position and harm our business prospects. In addition, we sublicense certain of our rights under our third-party licenses to BI and may sublicense such rights to current or future collaborators. Any impairment of these sublicensed rights could result in reduced revenue under our collaboration agreement with BI or result in termination of an agreement by one or more of our existing or any other future collaborators.

We may be unable to protect our intellectual property rights throughout the world.

Obtaining a valid and enforceable issued or granted patent covering our technology in the U.S. and worldwide can be extremely costly. In jurisdictions where we have not obtained patent protection, competitors may use our technology to develop their own products, and further, may export otherwise infringing products to territories where we have patent protection, but where it is more difficult to enforce a patent compared to the U.S. We also may face competition in jurisdictions where we do not have issued or granted patents or where our issued or granted patent claims or other intellectual property rights are not sufficient to prevent competitor activities in these jurisdictions. The legal systems of certain countries, particularly certain developing countries, make it difficult to enforce patents and such countries may not recognize other types of intellectual property protection, particularly that relating to biopharmaceuticals. This could make it difficult for us to prevent the infringement of our patents or marketing of competing products in violation of our proprietary rights generally in certain jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

We generally file a provisional patent application first (a priority filing) at the USPTO. A U.S. utility application and/or international application under the Patent Cooperation Treaty (“PCT”) are usually filed within 12 months after the priority filing. Based on the PCT filing, national and regional patent applications may be filed in the EU, Japan, Australia, and Canada and, depending on the individual case, also in any or all of, inter alia, China, India, South

Korea, Singapore, Taiwan, and South Africa. We have so far not filed for patent protection in all national and regional jurisdictions where such protection may be available. In addition, we may decide to abandon national and regional patent applications before grant. Finally, the grant proceeding of each national or regional patent is an independent proceeding which may lead to situations in which applications might be refused in some jurisdictions, while granted by others. Depending on the country, various scopes of patent protection may be granted on the same product candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws in the U.S., and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. If we or our licensors encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important

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for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition from others in those jurisdictions. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position in the relevant jurisdiction may be impaired and our business and results of operations may be adversely affected.

We, our licensors, or existing or future collaborators may become subject to third-party claims or litigation alleging infringement of patents or other proprietary rights or seeking to invalidate patents or other proprietary rights, and we may need to resort to litigation to protect or enforce our patents or other proprietary rights, all of which could be costly, time consuming, delay, or prevent the development and commercialization of our product candidates, or put our patents and other proprietary rights at risk.

We, our licensors, or existing or future collaborators may be subject to third-party claims for infringement or misappropriation of patent or other proprietary rights. We are generally obligated under our license or collaboration agreements to indemnify and hold harmless our licensors or collaborators for damages arising from intellectual property infringement by us. If we, our licensors, or existing or future collaborators are found to infringe a third-party patent or other intellectual property rights, we could be required to pay damages, potentially including treble damages, if we are found to have willfully infringed. In addition, we, our licensors, or existing or future collaborators may choose to seek, or be required to seek, a license from a third party, which may not be available on acceptable terms, if at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. If we fail to obtain a required license, we, our licensors, or existing or future collaborators may be unable to effectively market product candidates based on our technology, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. In addition, we may find it necessary to pursue claims or initiate lawsuits to protect or enforce our patent or other intellectual property rights. The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

If we were to initiate legal proceedings against a third party to enforce a patent covering one of our products or our technology, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness, 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 patent prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during patent prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our products or certain aspects of our platform technology. Such a loss of patent protection could have a material adverse impact on our business. Patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without legally infringing our patents or other intellectual property rights.

If we fail to comply with our obligations under any license, collaboration, or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our product candidates and delivery technologies, or we could lose certain rights to grant sublicenses.

Any future licenses we enter are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement, and other obligations on us. If we

breach any of these obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages, and the licensor may have the right to terminate the license, which could result in us being unable to develop, manufacture, and sell products that are covered by the licensed technology, or enable a competitor to gain access to the licensed technology. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

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

In addition to seeking patent protection for certain aspects of our product candidates and delivery technologies, we also consider trade secrets, including confidential and unpatented know-how, important to the maintenance of our competitive position. We protect trade secrets and confidential and unpatented know-how, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to such knowledge, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors, and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants that obligate them to maintain confidentiality and assign their inventions to us. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, some courts in the U.S. and certain foreign jurisdictions are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We are also subject both in the U.S. and outside the U.S. to various regulatory schemes regarding requests for the information we provide to regulatory authorities, which may include, in whole or in part, trade secrets or confidential commercial information. While we are likely to be notified in advance of any disclosure of such information and would likely object to such disclosure, there can be no assurance that our challenge to the request would be successful.

We may be, in the future, subject to claims that we or our employees or consultants have wrongfully used or disclosed alleged trade secrets of our employees' or consultants' former employers or their clients. These claims may be costly to defend and if we do not successfully do so, we may be required to pay monetary damages, may be prohibited from using some of our research and development work, and may lose valuable intellectual property rights or personnel.

Many of our employees were previously employed at universities or biotechnology or pharmaceutical companies, including our competitors or potential competitors. From time to time, we have received correspondence from other companies alleging the improper use or disclosure, or inquiring regarding the use or disclosure, by certain of our employees who have previously been employed elsewhere in our industry, including with our competitors, of their former employer's trade secrets or other proprietary information. Responding to these allegations can be costly and disruptive to our business, even when the allegations are without merit, and can be a distraction to management.

We may be subject to additional claims in the future that these or other employees of the Company have, or we have, inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending current or future claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, personnel, or the ability to use some of our research and development work. A loss of intellectual property, key research personnel, or their work product could hamper our ability to commercialize, or prevent us from commercializing, our product candidates, which could severely harm our business.

If our trademarks and trade names are not adequately protected, we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. Any trademark litigation could be expensive. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential collaborators or customers in our markets of interest. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Risks Related to Government Regulation

We may be unable to obtain U.S. or foreign regulatory approval and, as a result, may be unable to commercialize our product candidates.

Our product candidates are subject to extensive governmental regulations relating to, among other things, research, development, testing, manufacture, quality control, approval, labeling, packaging, promotion, storage, record-keeping, advertising, distribution, sampling, pricing, sales and marketing, safety, post-approval monitoring and reporting, and export and import of drugs. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process are required to be successfully completed in the U.S. and in many foreign jurisdictions before a new drug can be marketed. Satisfaction of these and other regulatory requirements is costly, time consuming, uncertain, and subject to unanticipated delays. It is possible that none of the product candidates we may develop will obtain the regulatory approvals necessary for us or our collaborators to begin selling them.

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We have very limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA as well as foreign regulatory authorities, such as the EMA. The time required to obtain FDA and foreign regulatory approvals is unpredictable but typically takes many years following the commencement of clinical trials, depending upon the type, complexity, and novelty of the product candidate. The standards that the FDA and its foreign counterparts use when regulating us are not always applied predictably or uniformly and can change. Any analysis we perform of data from preclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit, or prevent regulatory approval. We may also encounter unexpected delays or increased costs due to new government regulations, for example, from future legislation or administrative action, or from changes in the policy of the FDA or foreign regulatory authorities during the period of product development, clinical trials, and regulatory review by the FDA or foreign regulatory authorities. It is impossible to predict whether legislative changes will be enacted, or whether FDA or foreign laws, regulations, guidance, or interpretations will be changed, or what the impact of such changes, if any, may be.

Because the drugs we are developing may represent a new class of drug, the FDA and its foreign counterparts have not yet established any definitive policies, practices, or guidelines in relation to these drugs. While we believe the product candidates that we are currently developing are regulated as new drugs under the Federal Food, Drug, and Cosmetic Act, the FDA could decide to reclassify them, namely to regulate them or other products we may develop as biologics under the Public Health Service Act. The lack of policies, practices, or guidelines may hinder or slow review by the FDA or foreign regulatory authorities of any regulatory filings that we may submit. Moreover, the FDA or foreign regulatory authorities may respond to these submissions by defining requirements we may not have anticipated. Such responses could lead to significant delays in the clinical development of our product candidates. In addition, because there may be approved treatments for some of the diseases for which we may seek approval, in order to receive regulatory approval, we may need to demonstrate through clinical trials that the product candidates we develop to treat these diseases, if any, are not only safe and effective, but safer or more effective than existing products.

Any delay or failure in obtaining required approvals could have a material adverse effect on our ability to generate revenues from the particular product candidate for which we are seeking approval. Furthermore, any regulatory approval to market a product may be subject to limitations on the approved uses for which we may market the product or the labeling or other restrictions. Regulatory authorities also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. In addition, the FDA has the authority to require a REMS plan as part of an NDA or biologics license application or after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug or biologic, such as limiting prescribing to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients who meet certain safe-use criteria, and requiring treated patients to enroll in a registry. These limitations and restrictions may limit the size of the market for the product and affect coverage and reimbursement by third-party payors.

We are also subject to numerous foreign regulatory requirements governing, among other things, the conduct of clinical trials, manufacturing and marketing authorization, pricing, and third-party reimbursement. The foreign regulatory approval process varies among countries and may include all of the risks associated with FDA approval described above as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Moreover, the time required to obtain approval may differ from that required to obtain FDA approval. Approval by the FDA does not ensure approval by regulatory authorities outside the U.S. and vice versa.

If we or current or future collaborators, manufacturers, or service providers fail to comply with healthcare laws and regulations, we or they could be subject to enforcement actions and substantial penalties, which could affect our ability to develop, market, and sell our products, and may harm our reputation.

Although we do not currently have any products on the market, once our therapeutic candidates or clinical trials are covered by federal healthcare programs, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal, state and foreign governments of the jurisdictions in which we conduct our business. Healthcare providers, physicians, and third-party payors play a primary role in the recommendation and prescription of any therapeutic candidates for which we obtain marketing approval. Our future arrangements with

third-party payors and customers may expose us to broadly applicable fraud and abuse, transparency, and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell, and distribute our therapeutic candidates for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include, but are not limited to, the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons from soliciting, receiving, offering, or providing remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce either the referral of an individual for a healthcare item or service, or the purchasing or ordering of an item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare or Medicaid;
- federal civil and criminal false claims laws and civil monetary penalty laws, such as the U.S. federal False Claims Act ("FCA"), which imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease, or conceal an obligation to pay money to the

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federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA;

- HIPAA includes a fraud and abuse provision referred to as the HIPAA All-Payor Fraud Law, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing, or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items, or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by HITECH, and its implementing regulations, which impose obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;
- federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the federal Physician Payment Sunshine Act and the implementing regulations, also referred to as "Open Payments," issued under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act of 2010, collectively referred to as the "ACA," which require that manufacturers of pharmaceutical and biological drugs reimbursable under Medicare, Medicaid, and Children's Health Insurance Programs report to the Department of Health and Human Services all consulting fees, travel reimbursements, research grants, and other payments, transfers of value, or gifts made to physicians and teaching hospitals with limited exceptions;
- analogous state laws and regulations, such as state anti-kickback and false claims laws potentially applicable to sales or marketing arrangements and claims involving healthcare items or services reimbursed by nongovernmental third-party payors, including private insurers; and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. Some states, such as California, Massachusetts and Vermont, mandate implementation of corporate compliance programs, along with the tracking and reporting of gifts, compensation, and other remuneration to physicians; and
- the EU General Data Protection Regulation ("GDPR"), which was officially adopted in April 2016 and went into effect in May 2018, introduces new data protection requirements in the EU, as well as substantial fines for breaches of the data protection rules. The GDPR enhances data protection obligations for processors and controllers of personal data, including, for example, expanded disclosures about how personal information is to be used, limitations on retention of information, mandatory data breach notification requirements and onerous new obligations on services providers. Non-compliance with the GDPR may result in monetary penalties of up to €20 million or 4% of worldwide revenue, whichever is higher. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of personal data, such as healthcare data or other sensitive information, could greatly increase our cost of providing our products and services or even prevent us from offering certain services in jurisdictions in which we operate.

The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions, and settlements in the healthcare industry. Responding to investigations can be time and resource-consuming and can divert management's attention from the business. Any such investigation or settlement could increase our costs or otherwise have an

adverse effect on our business.

Ensuring that our business arrangements with third parties comply with applicable healthcare laws and regulations could involve substantial costs. If our operations are found to be in violation of any such requirements, we may be subject to penalties, including civil or criminal penalties, monetary damages, the curtailment or restructuring of our operations, or exclusion from participation in government contracting, healthcare reimbursement, or other government programs, including Medicare and Medicaid, any of which could adversely affect our financial results. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. In addition, achieving and sustaining compliance with applicable laws and regulations may be costly to us in terms of money, time, and resources.

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If we or current or future collaborators, manufacturers, or service providers fail to comply with applicable federal, state, or foreign laws or regulations, we could be subject to enforcement actions, which could affect our ability to develop, market, and sell our products successfully and could harm our reputation and lead to reduced acceptance of our products by the market. These enforcement actions include, among others:

- adverse regulatory inspection findings;
- warning or untitled letters;
- voluntary or mandatory product recalls or public notification or medical product safety alerts to healthcare professionals;
- restrictions on, or prohibitions against, marketing our products;
- restrictions on, or prohibitions against, importation or exportation of our products;
- suspension of review or refusal to approve pending applications or supplements to approved applications;
- exclusion from participation in government-funded healthcare programs;
- exclusion from eligibility for the award of government contracts for our products;
- a corporate integrity agreement;
- FDA debarment of individuals at our Company;
- suspension or withdrawal of product approvals;
- seizure or administrative detention of products;
- injunctions; and
- civil and criminal penalties and fines.

Any drugs we develop may become subject to unfavorable pricing regulations, third-party coverage, and reimbursement practices or healthcare reform initiatives, thereby harming our business.

The regulations that govern marketing approvals, pricing, coverage, and reimbursement for new drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. Although we intend to monitor these regulations, our programs are currently in the early stages of development and we will not be able to assess the impact of price regulations for a number of years. As a result, we might obtain regulatory approval for a product in a particular country but then be subject to price regulations that delay our commercial launch of the product and negatively impact the revenues we are able to generate from the sale of the product in that country.

Our ability to commercialize any products successfully will also depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, and other organizations. However, there may be significant delays in obtaining coverage for newly-approved drugs. Moreover, eligibility for coverage does not necessarily signify that a drug will be reimbursed in all cases or at a rate that covers our costs, including research, development, manufacture, sale, and distribution costs. Also, interim payments for new drugs, if applicable, may be insufficient to cover our costs and may not be made permanent. Thus, even if we succeed in bringing one or more products to the market, these products may not be considered medically necessary or cost-effective, and the amount reimbursed for any products may be insufficient to allow us to sell our products on a competitive basis. Because our programs are in the early stages of development, we are unable at this time to determine their cost effectiveness, or the likely level or method of reimbursement. In addition, obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to each payor supporting scientific, clinical, and cost-effectiveness data for the use of our product on a payor-by-payor basis, with no assurance that coverage and adequate reimbursement will be obtained. A payor’s decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor’s determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize any product candidate that we successfully develop.

Increasingly, the third-party payors who reimburse patients or healthcare providers, such as government and private insurance plans, are seeking greater upfront discounts, additional rebates, and other concessions to reduce the prices for pharmaceutical products. If the price we are able to charge for any products we develop, or the reimbursement provided for such products, is inadequate in light of our development and other costs, our return on investment could be adversely affected.

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We currently expect that certain drugs we develop may need to be administered under the supervision of a physician on an outpatient basis. Under currently applicable U.S. law, certain drugs that are not usually self-administered (including injectable drugs) may be eligible for coverage under Medicare through Medicare Part B. Specifically, Medicare Part B coverage may be available for eligible beneficiaries when the following, among other requirements have been satisfied:

- the product is reasonable and necessary for the diagnosis or treatment of the illness or injury for which the product is administered according to accepted standards of medical practice;
- the product is typically furnished incident to a physician's services;
- the indication for which the product will be used is included or approved for inclusion in certain Medicare-designated pharmaceutical compendia (when used for an off-label use); and
- the product has been approved by the FDA.

Average prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. Reimbursement rates under Medicare Part B would depend in part on whether the newly approved product would be eligible for a unique billing code. Self-administered, outpatient drugs are typically reimbursed under Medicare Part D, and drugs that are administered in an inpatient hospital setting are typically reimbursed under Medicare Part A under a bundled payment. It is difficult for us to predict how Medicare coverage and reimbursement policies will be applied to our products in the future and coverage and reimbursement under different federal healthcare programs are not always consistent. Medicare reimbursement rates may also reflect budgetary constraints placed on the Medicare program.

Third-party payors often rely upon Medicare coverage policies and payment limitations in setting their own reimbursement rates. These coverage policies and limitations may rely, in part, on compendia listings for approved therapeutics. Our inability to promptly obtain relevant compendia listings, coverage, and adequate reimbursement from both government-funded and private payors for new drugs that we develop and for which we obtain regulatory approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products, and our financial condition.

We expect that these and other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and lower reimbursement, and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government-funded programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs once marketing approval is obtained.

We believe that the efforts of governments and third-party payors to contain or reduce the cost of healthcare and legislative and regulatory proposals to broaden the availability of healthcare will continue to affect the business and financial condition of pharmaceutical and biopharmaceutical companies. A number of legislative and regulatory changes in the healthcare system in the U.S. and other major healthcare markets have been proposed, and such efforts have expanded substantially in recent years. These developments could, directly or indirectly, affect our ability to sell our products, if approved, at a favorable price.

For example, in the U.S., in 2010, the U.S. Congress passed the ACA, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of health spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry, and impose additional policy reforms. Both the U.S. Congress and President Trump have expressed an intention to repeal or replace the ACA, and as a result, certain sections of the ACA have not been fully implemented or have been effectively repealed. The uncertainty around the future of the ACA and, in particular, the impact to reimbursement levels, may lead to uncertainty or delay in the purchasing decisions of our customers, which may in turn negatively impact our product sales. If there are not adequate reimbursement levels, our business and results of operations could be adversely affected.

Among the provisions of the ACA addressing coverage and reimbursement of pharmaceutical products, of importance to our potential therapeutic candidates are the following

- increases to pharmaceutical manufacturer rebate liability under the Medicaid Drug Rebate Program due to an increase in the minimum basic Medicaid rebate on most branded prescription drugs and the application of Medicaid rebate liability to drugs used in risk-based Medicaid managed care plans;
- the expansion of the 340B Drug Pricing Program to require discounts for “covered outpatient drugs” sold to certain children’s hospitals, critical access hospitals, freestanding cancer hospitals, rural referral centers, and sole community hospitals;

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- requirements imposed on pharmaceutical companies to offer discounts on brand-name drugs to patients who fall within the Medicare Part D coverage gap, commonly referred to as the "Donut Hole";
- requirements imposed on pharmaceutical companies to pay an annual non-tax-deductible fee to the federal government based on each company's market share of prior year total sales of branded drugs to certain federal healthcare programs, such as Medicare, Medicaid, Department of Veterans Affairs, and Department of Defense. Since we currently expect our branded pharmaceutical sales to constitute a small portion of the total federal healthcare program pharmaceutical market, we do not currently expect this annual assessment to have a material impact on our financial condition; and
- For products classified as biologics, marketing approval for a follow-on biologic product may not become effective until 12 years after the date on which the reference innovator biologic product was first licensed by the FDA, with a possible six-month extension for pediatric products. After this exclusivity ends, it may be possible for biosimilar manufacturers to enter the market, which is likely to reduce the pricing for the innovator product and could affect our profitability if our products are classified as biologics.

Despite initiatives to invalidate the Affordable Care Act, the U.S. Supreme Court has upheld certain key aspects of the legislation, including a tax-based shared responsibility payment imposed on certain individuals who fail to maintain qualifying health coverage for all or part of a year, which is commonly referred to as the "individual mandate." However, as a result of tax reform legislation passed in December 2017, the individual mandate has been eliminated effective January 1, 2019. On December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the ACA are invalid as well. The Trump administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018, the same judge issued an order staying the judgment pending appeal. It is unclear how this decision and any subsequent appeals and other efforts to repeal and replace the ACA will impact the ACA and our business. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results. We will continue to evaluate the effect that the ACA and its possible repeal and replacement has on our business.

In addition, since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Further, the Trump administration has concluded that Cost-Sharing Reduction ("CSR") payments to insurance companies required under the ACA have not received necessary appropriations from Congress and announced that it will discontinue these payments immediately until such appropriations are made. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017 and again on July 18, 2018. Furthermore, on June 14, 2018, the U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in ACA risk corridor payments to third-party payors who argued were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the ACA marketplace, providers, and potentially our business, are not yet known.

Also, in 2018, the Right to Try Act of 2018 provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a drug manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act, but the manufacturer must develop an internal policy and respond to patient requests according to that policy.

From time to time, legislation is drafted, introduced, and passed in the U.S. Congress that could significantly change the statutory provisions governing coverage, reimbursement, and marketing of products regulated by CMS or other government agencies. In addition to new legislation, CMS coverage and reimbursement policies are often revised or interpreted in ways that may significantly affect our business and our products.

The healthcare industry is heavily regulated in the U.S. at the federal, state, and local levels, and our failure to comply with applicable requirements may subject us to penalties and negatively affect our financial condition.

As a healthcare company, our operations, clinical trial activities, and interactions with healthcare providers may be subject to extensive regulation in the U.S., particularly if we receive FDA approval for any of our products in the future. For example, if we receive FDA approval for a product for which reimbursement is available under a federal healthcare program (e.g., Medicare, Medicaid), it would be subject to a variety of federal laws and regulations, including those that prohibit the filing of false or improper claims for payment by federal healthcare programs (e.g., the federal FCA), prohibit unlawful inducements for the referral of business reimbursable by federal healthcare programs (e.g., the federal Anti-Kickback Statute), and require disclosure of certain payments or other transfers of value made to U.S.-licensed physicians and teaching hospitals or Open Payments. We are not able to predict how

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third parties will interpret these laws and apply applicable governmental guidance and may challenge our practices and activities under one or more of these laws. If our past or present operations are found to be in violation of any of these laws, we could be subject to civil and criminal penalties which could hurt our business, our operations, and financial condition.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity from knowingly and willfully offering, paying, soliciting, or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for, purchasing, leasing, ordering, or arranging for the purchase, lease, or order of any item or service reimbursable under Medicare, Medicaid, or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. 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, and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing, or recommending 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 of its facts and circumstances. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor.

Additionally, the intent standard under the Anti-Kickback Statute was amended by the ACA to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the ACA codified case law 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 FCA (discussed below).

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 healthcare program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Federal false claims and false statement laws, including the federal FCA, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment to, or approval by, the federal healthcare programs, including Medicare and Medicaid, or knowingly making, using, or causing to be made or used, a false record or statement material to a false or fraudulent claim to the federal government. A claim includes “any request or demand” for money or property presented to the U.S. government. For instance, historically, pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies’ marketing of the product for unapproved, off-label, and thus generally non-reimbursable, uses.

HIPAA prohibits, among other offenses, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private payors, or 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 items or services under a healthcare benefit program. To the extent that we act as a business associate to a healthcare provider engaging in electronic transactions, we may also be subject to the privacy and security provisions of HIPAA, as amended by HITECH, which restricts the use and disclosure of patient-identifiable health information, mandates the adoption of standards relating to the privacy and security of patient-identifiable health information, and requires the reporting of certain security breaches to healthcare provider customers with respect to such information. Additionally, many states have enacted similar laws that may impose more stringent requirements on entities like ours. Failure to comply with applicable laws and regulations could result in substantial penalties and adversely affect our financial condition and results of operations.

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

Certain Dicerna products, if approved, may be eligible for coverage under Medicare and Medicaid, among other government healthcare programs. Accordingly, Dicerna may be subject to a number of obligations based on its participation in these programs, such as a requirement to calculate and report certain price reporting metrics to the government, such as average sales price and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S. It is difficult to predict how Medicare coverage and reimbursement policies will be applied to Dicerna's products in the future, and coverage and reimbursement under different federal healthcare programs are not always consistent. Medicare reimbursement rates may also reflect budgetary constraints placed on the Medicare program.

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In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical and biotechnology companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, civil, criminal, and/or administrative penalties, damages, fines, disgorgement, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, private "qui tam" actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, 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.

Our ability to obtain reimbursement or funding from the federal government may be impacted by possible reductions in federal spending.

U.S. federal government agencies currently face potentially significant spending reductions. The Budget Control Act of 2011 (the "BCA") established a Joint Select Committee on Deficit Reduction, which was tasked with achieving a reduction in the federal debt level of at least $1.2 trillion. That committee did not draft a proposal by the BCA's deadline. As a result, automatic cuts, referred to as sequestration, in various federal programs were scheduled to take place, beginning in January 2013, although the American Taxpayer Relief Act of 2012 delayed the BCA's automatic cuts until March 1, 2013. While the Medicare program's eligibility and scope of benefits are generally exempt from these cuts, Medicare payments to providers and Part D health plans are not exempt. The BCA did, however, provide that the Medicare cuts to providers and Part D health plans would not exceed two percent. President Obama issued the sequestration order on March 1, 2013, and cuts went into effect on April 1, 2013. Additionally, the Bipartisan Budget Act of 2015 extended sequestration for Medicare through fiscal year 2027.

The U.S. federal budget remains in flux, which could, among other things, cut Medicare payments to providers. The Medicare program is frequently mentioned as a target for spending cuts. The full impact on our business of any future cuts in Medicare or other programs is uncertain. In addition, we cannot predict any impact President Trump's administration and the U.S. Congress may have on the federal budget. If federal spending is reduced, anticipated budgetary shortfalls may also impact the ability of relevant agencies, such as the FDA or the National Institutes of Health, to continue to function at current levels. Amounts allocated to federal grants and contracts may be reduced or eliminated. These reductions may also impact the ability of relevant agencies to timely review and approve drug research and development, manufacturing, and marketing activities, which may delay our ability to develop, market, and sell any products we may develop.

If any of our product candidates receive marketing approval and we or others later identify undesirable side effects caused by the product candidate, our ability to market and derive revenue from the product candidates could be compromised.

In the event that any of our product candidates receive regulatory approval and we or others identify undesirable side effects, adverse events, or other problems caused by one of our products, any of the following adverse events could occur, which could result in the loss of significant revenue to us and materially and adversely affect our results of operations and business:

- regulatory authorities may withdraw their approval of the product or seize the product;

- we may need to recall the product or change the way the product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing processes for the product or any component thereof;
- we may not be able to secure or maintain adequate coverage and reimbursement for our proprietary product candidates from government (including U.S. federal healthcare programs) and private payors;
- we may be subject to fines, restitution or disgorgement of profits or revenues, injunctions, or the imposition of civil penalties or criminal prosecution;

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- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;
- regulatory authorities may require us to implement a REMS, or to conduct post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product; we may be required to create a Medication Guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

Risks Related to Our Common Stock

We are an “emerging growth company” and a “smaller reporting company,” and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies will make our common stock less attractive to investors.

We are an “emerging growth company” as defined in the Jumpstart Our Business Startups Act (“JOBS Act”). For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including (1) not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act of 2002 (“Sarbanes-Oxley Act”), (2) reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and (3) exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of the prior June 30 or if we have total annual gross revenue of $1.07 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31, or if we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, in which case we would no longer be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may, under certain circumstances, still qualify as a “smaller reporting company,” which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404(b) of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be more volatile.

Our stock price is volatile and purchasers of our common stock could incur substantial losses.

Our stock price has historically fluctuated widely and is likely to continue to be volatile. From January 30, 2014, the first day of trading of our common stock, through March 4, 2019, the closing sale price of our common stock has ranged between a high of $46.00 per share and a low of $2.45 per share. The market price for our common stock may be influenced by many factors, including the other risks described in this “Risk Factors” section, and the following:

- the success or failure of competitive products or technologies;
- delays in initiating or completing and the results of preclinical studies and clinical trials of our product candidates, or those of our competitors, our existing collaborator, or any future collaborators;
- regulatory or legal developments in the U.S. and other countries, especially changes in laws or regulations applicable to our product candidates;
- introductions and announcements of new products by us, our commercialization collaborators, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our or our competitors’ product candidates, products, clinical studies, manufacturing process, or sales and marketing terms;
- actual or anticipated variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our or our competitors’ efforts to acquire or in-license additional technologies, products, or product candidates;

- developments concerning our or our competitors' collaborations, including but not limited to, those with sources of manufacturing supply and commercialization partners;

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- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures, or capital commitments;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies, or our industry generally;
- our failure or the failure of our competitors to meet analysts' projections or guidance that we or our competitors may give to the market;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- announcement and expectation of additional financing efforts;
- speculation in the press or investment community;
- trading volume of our common stock;
- sales of our common stock by us or our stockholders;
- the absence of lock-up agreements with the holders of substantially all of our outstanding shares in connection with follow-on public offerings of our common stock;
- the concentrated ownership of our common stock;
- changes in accounting principles;
- terrorist acts, acts of war, or periods of widespread civil unrest;
- natural disasters and other calamities;
- general economic, industry, and market conditions; and
- developments concerning complaints or litigation against us.

In addition, the stock markets in general, and the markets for pharmaceutical, biopharmaceutical, and biotechnology stocks in particular have experienced extreme volatility that has often been unrelated to the operating performance of the issuer. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our operating performance.

The future issuance of equity or of debt securities that are convertible into equity will dilute our share capital.

We may choose to raise additional capital in the future depending on market conditions, strategic considerations, and operational requirements. To the extent that additional capital is raised through the issuance of shares or other securities convertible into shares, our stockholders will be diluted. Future issuances of our common stock or other equity securities, or the perception that such sales may occur, could adversely affect the trading price of our common stock and impair our ability to raise capital through future offerings of shares or equity securities. We cannot predict the effect, if any, that future sales of common stock or the availability of common stock for future sales will have on the trading price of our common stock.

The employment agreements with our executive officers may require us to pay severance benefits to officers who are terminated in connection with a change of control of the Company, which could harm our financial condition.

Our executive officers are parties to employment agreements providing, in the event of a termination of employment in connection with a change of control of the Company, for significant cash payments for severance and other benefits and acceleration of vesting of up to all outstanding stock options. The accelerated vesting of options could result in dilution to our existing stockholders and reduce the market price of our common stock. The payment of these severance benefits could harm our financial condition. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

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 stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. If any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business

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model, our intellectual property, or our stock performance, or if our target studies and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause our stock price or trading volume to decline.

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

As of December 31, 2018, our executive officers and directors, together with holders of five percent or more of our outstanding common stock and their respective affiliates, beneficially owned, in the aggregate, approximately 53% of our outstanding common stock, including shares subject to outstanding options and warrants that are exercisable within 60 days after such date, based on the Forms 3 and 4 and Schedules 13D and 13G filed by them with the SEC. As a result, these stockholders, if acting together, will continue to have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, any merger, consolidation, or sale of all or substantially all of our assets, and any other significant corporate transaction. The interests of these stockholders may not be the same as, or may even conflict with, the interests of our other stockholders. For example, these stockholders could delay or prevent a change of control of our Company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our Company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

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

Provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may delay or prevent an acquisition of us or a change in our management. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Because our board of directors is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. These provisions include:

- a prohibition on actions by our stockholders by written consent;
- a requirement that special meetings of stockholders, which the Company is not obligated to call more than once per calendar year, be called only by the chairman of our board of directors, our chief executive officer, our board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors, or, subject to certain conditions, by our secretary at the request of the stockholders holding of record, in the aggregate, shares entitled to cast not less than ten percent of the votes at a meeting of the stockholders (assuming all shares entitled to vote at such meeting were present and voted);
- advance notice requirements for election to our board of directors and for proposing matters that can be acted upon at stockholder meetings; and
- the authority of the board of directors to issue preferred stock, such as the Redeemable Convertible Preferred, with such terms as the board of directors may determine.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, as amended, which prohibits a person who owns in excess of 15 percent of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15 percent of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. These provisions would apply even if the proposed merger or acquisition could be considered beneficial by some stockholders.

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

As a public company, we incur, and particularly after we are no longer an emerging growth company and when we cease to be a smaller reporting company, we will continue to incur significant legal, accounting, and other expenses

that we did not incur as an emerging growth company or smaller reporting company.

The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The Nasdaq Global Select Market, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our

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management and other personnel need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, we expect that these rules and regulations make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors. However, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

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

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be sole source of gain of our common stockholders for the foreseeable future.

We may incur significant costs from class action litigation due to our historical or expected stock volatility.

Our stock price has fluctuated and may fluctuate for many reasons, including as a result of public announcements regarding the progress of our development efforts or the development efforts of our collaborators or competitors, the addition or departure of our key personnel, variations in our quarterly operating results, and changes in market valuations of pharmaceutical and biotechnology companies. This risk is especially relevant to us because pharmaceutical and biotechnology companies have experienced significant stock price volatility in recent years. When the market price of a stock has been volatile as our stock price has been and may be, holders of that stock have occasionally brought securities class action litigation against the company that issued the stock. If any of our stockholders were to bring a lawsuit of this type against us, even if the lawsuit is without merit, we could incur substantial costs defending the lawsuit. The lawsuit could also divert the time and attention of our management.

Our amended and restated bylaws designate the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our amended and restated bylaws provide that, subject to limited exceptions, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, or other employees to us or our stockholders, any action asserting a claim against us arising pursuant to any provision of the Delaware General Corporation Law, as amended, our amended and restated certificate of incorporation or our amended and restated bylaws, any action to interpret, apply, enforce, or determine the validity of our amended and restated certificate of

incorporation or our amended and restated bylaws, any action to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws, or any other action asserting a claim against us that is governed by the internal affairs doctrine. Any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock shall be deemed to have notice of and to have consented to the provisions of our amended and restated certificate of incorporation described above. This 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 us and our directors, officers, and employees. Alternatively, if a court were to find these provisions of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types

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of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could adversely affect our business and financial condition.

Our stockholders may experience significant dilution as a result of future equity offerings and exercise of outstanding options.

In order to raise additional capital, we may in the future offer additional shares of our common stock or other securities convertible into or exchangeable for our common stock, as we did with the Redeemable Convertible Preferred, which was converted into common stock in December 2017 and with the follow-on offerings of our common stock in December 2017 and September 2018. We cannot assure you that we will be able to sell shares or other securities in any offering at a price per share that is equal to or greater than the price paid by our existing shareholders, and investors purchasing shares or other securities in the future could have rights superior to existing stockholders. The price per share at which we sell additional shares of our common stock or other securities convertible into or exchangeable for our common stock in future transactions may be higher or lower than the price per share paid by our existing stockholders.

In addition, we have a significant number of securities allowing the purchase of our common stock. As of March 4, 2019, we also had 3,733,792 shares of common stock reserved for future issuance under our stock incentive plans. As of that date, there were also stock options and awards to purchase 11,206,340 shares of our common stock outstanding and warrants to purchase 2,198 shares of our common stock outstanding. The exercise of outstanding options and warrants having an exercise price per share that is less than the offering price per share paid by our existing stockholders will increase dilution to such stockholders.

Future sales of our common stock in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. As of March 4, 2019, we had 68,264,949 shares of common stock outstanding, all of which, other than shares held by our collaboration partners, Alexion and Lilly, which are subject to certain lock-up provisions, and shares held by our directors and certain officers, were eligible for sale in the public market, subject in some cases to compliance with the requirements of Rule 144, including the volume limitations and manner of sale requirements. In addition, shares of common stock issuable upon exercise of outstanding options and shares reserved for future issuances under our stock incentive plans will become eligible for sale in the public market to the extent permitted by applicable vesting requirements and subject in some cases to compliance with the requirements of Rule 144.

Sales of shares issued in private placements may cause the market price of our shares to decline.

In April 2017, we issued 700,000 shares of the Redeemable Convertible Preferred in a private placement, which were convertible into shares of our common stock at an agreed conversion rate. In December 2017, all shares of Redeemable Convertible Preferred were converted into shares of our common stock. We granted the holders of Redeemable Convertible Preferred certain demand, shelf, and “piggyback” registration rights with respect to the shares of common stock issued upon conversion of the Redeemable Convertible Preferred. Such registration rights continue subsequent to the conversion and repurchase of the Redeemable Convertible Preferred with respect to the shares of common stock issued in such conversion. In accordance with such registration rights, we filed a shelf registration statement on Form S-3 covering the resale of 24,491,663 shares of our common stock by the former holders of Redeemable Convertible Preferred. The registration statement was declared effective on May 9, 2018, and all shares of common stock issued upon conversion of the Redeemable Convertible Preferred may now be freely sold in the open market. Additionally, we issued 983,208 shares of our common stock to Alnylam in April 2018, 835,834 shares of our common stock to Alexion in October 2018, and 5,414,185 shares of our common stock to Lilly in December 2018. The shares issued to Alnylam are freely tradeable in the open market, subject to certain volume limitations and compliance with applicable securities laws. The shares issued to Alexion and Lilly are subject to a lock-up period, but following the expiration of such lock-up periods, such shares of our common stock may be freely sold in the open market, subject to compliance with applicable securities laws. The sale of a significant amount of these shares in the open market or the perception that these sales may occur could cause the market price of our common stock to decline or become highly volatile.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

Our corporate headquarters are located in Cambridge, Massachusetts, where we lease 37,084 square feet of office and laboratory space. The lease term for our office and laboratory space in Cambridge, Massachusetts, commenced in December 2014 for a lease term of six years.

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On January 2, 2019, we entered into a seven-year term lease for 80,872 square feet of office and laboratory space located in Lexington, Massachusetts ("Lexington"). The location in Lexington will become the Company's corporate headquarters upon occupancy, currently anticipated to occur in the fourth quarter of 2019.

We believe that suitable additional or alternative space will be available as needed on commercially reasonable terms.

ITEM 3. LEGAL PROCEEDINGS

From time to time we may be subject to legal proceedings, claims, and litigation arising in the ordinary course of business. We are not currently a party to or aware of any proceedings that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition, or results of operations. Any future litigation could result in substantial costs and divert our management's attention and resources, which could cause serious harm to our business, operating results, and financial condition. We maintain liability insurance; however, if any costs or expenses associated with this or any other litigation exceed our insurance coverage, we may be forced to bear some or all of these costs and expenses directly, which could be substantial.

Settlement of Alnylam Litigation

On June 10, 2015, Alnylam Pharmaceuticals, Inc. ("Alnylam") filed a trade secret misappropriation lawsuit against us in the Superior Court of Middlesex County, Massachusetts seeking to enjoin Dicerna from the use of what they perceived as proprietary and trade secret information related to ribonucleic acid interference assets that Alnylam had purchased from Merck. The suit sought permanent injunctive relief and monetary damages from us. In August 2017, we filed counterclaims in a trade secret lawsuit alleging that the Alnylam actions represented abuse of process and claimed tortious interference with our business opportunities. In September 2017, Alnylam filed a motion to dismiss Dicerna's counterclaims and the motion was denied. In August 2017, we filed a lawsuit against Alnylam in the United States District Court of Massachusetts charging a monopolization violation of the Sherman Antitrust Act. In October 2017, Alnylam filed a motion to dismiss the antitrust lawsuit which was denied.

On April 18, 2018, the parties executed a Settlement & Release Agreement ("Settlement Agreement") resolving all ongoing litigation between the companies. The terms of the Settlement Agreement include mutual releases and dismissal with prejudice of all claims and counterclaims in the following litigation between the parties: (i) Alnylam Pharmaceuticals, Inc. v. Dicerna Pharmaceuticals, Inc., No. 15-4126, pending in the Massachusetts Superior Court for Middlesex County; and, (ii) Dicerna Pharmaceuticals, Inc. v. Alnylam Pharmaceuticals, Inc., pending in the United States District Court for the District of Massachusetts.

Under the terms of the Settlement Agreement, Dicerna paid an aggregate of $25.3 million including an upfront cash payment of $2.0 million and 983,208 shares of Dicerna common stock, valued at $10.3 million, that were initially received by Alnylam in the second quarter of 2018, with an additional $2.5 million paid in November 2018, and the final payment of $10.5 million paid in January 2019. In addition, Dicerna will be subject to time limitations on the development of certain gene targets for period ranging from 18 months up to four years. The Settlement Agreement does not provide any admission of wrongdoing by either company and does not provide any intellectual property licenses to either party.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

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PART II

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

Market Information for Common Stock

Our common stock trades on The Nasdaq Global Select Market under the symbol "DRNA." The following table sets forth the high and low sale prices per share for our common stock on The Nasdaq Global Select Market for the periods indicated:

Year Ended December 31, 2018	High	Low
First Quarter	$15.00	$8.16
Second Quarter	$15.80	$8.71
Third Quarter	$17.98	$11.66
Fourth Quarter	$16.06	$9.31
Year Ended December 31, 2017	High	Low
First Quarter	$3.40	$2.42
Second Quarter	$3.85	$2.87
Third Quarter	$5.82	$2.69
Fourth Quarter	$10.24	$4.91

Holders of Record

As of March 4, 2019, there were approximately 17 holders of record of our common stock. Because many of our shares of common stock are held by brokers and other institutions on behalf of stockholders, we are unable to estimate the total number of beneficial stockholders represented by these record holders.

Dividend Policy

We currently intend to retain future earnings, if any, for use in the operation of our business and to fund future growth. We have never declared or paid cash dividends on our common stock and we do not intend to pay any cash dividends on our common stock for the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors in light of conditions then existing, including factors such as our results of operations, financial condition and requirements, business conditions, and covenants under any applicable contractual arrangements.

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Performance Graph

The following graph illustrates a comparison of the total cumulative stockholder return on our common stock since January 30, 2014 (the date our stock became publicly traded on The Nasdaq Global Select Market) to The Nasdaq Composite and The Nasdaq Biotechnology indices. The graph assumes an initial investment of $100 on January 30, 2014. The stock price performance on the following graph is not necessarily indicative of future stock price performance. This performance graph shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or incorporated by reference into any of our filings under the Securities Act or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Recent Sales of Unregistered Securities

Not applicable.

Use of Proceeds from Initial Public Offering of Common Stock

Not applicable.

Purchases of Equity Securities by the Issuer and Affiliated Parties

None.

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ITEM 6. SELECTED FINANCIAL DATA

The following selected consolidated financial data has been derived from our audited consolidated financial statements. Our audited consolidated financial statements as of December 31, 2018 and 2017 and for the fiscal years ended December 31, 2018, 2017, and 2016 are included elsewhere in this Annual Report on Form 10-K. The information set forth below should be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations," included in Item 7 of this Annual Report on Form 10-K, and with our consolidated financial statements and notes thereto, included in Item 8 of this Annual Report on Form 10-K. The information set forth below is not necessarily indicative of our future results of operations or financial condition.

	YEAR ENDED DECEMBER 31,				
	2018	2017 [a]	2016	2015	2014
Results of operations data					
Revenue from collaborative arrangements [b]	$6,176	$1,030	$—	$—	$—
Net loss	$(88,853)	$(60,200)	$(59,513)	$(62,839)	$(47,939)
Net loss attributable to common stockholders	$(88,853)	$(80,292)	$(59,513)	$(62,839)	$(48,143)
Net loss per share attributable to common stockholders – basic and diluted	$(1.60)	$(3.66)	$(2.87)	$(3.09)	$(3.00)
Weighted average common shares outstanding – basic and diluted	55,616,092	21,917,415	20,719,761	20,320,628	16,070,054

(a) Reflects the retrospective adoption of the new revenue recognition accounting standard, which the Company adopted on January 1, 2018. Refer to Note 2 to our consolidated financial statements (see Item 8 of this Annual Report on Form 10-K) for more information.

(b) In 2018, we reclassified grant revenues of $1.1 million, $0.3 million, and $0.2 million as offsets to research and development expenses for the years ended December 31, 2017, 2016, and 2015, respectively, to conform to the current year's presentation.

	DECEMBER 31,				
	2018	2017 [a]	2016	2015	2014
Financial condition data					
Cash and cash equivalents	$54,239	$68,789	$20,865	$56,058	$26,067
Held-to-maturity investments	$248,387	$44,889	$25,009	$38,551	$72,556
Total assets	$409,041	$121,002	$51,252	$100,023	$103,605
Total noncurrent liabilities	$114,293	$3,090	$—	$—	$—
Total stockholders' equity	$200,693	$101,086	$41,208	$91,022	$98,340

(a) Reflects the retrospective adoption of the new revenue recognition accounting standard, which the Company adopted on January 1, 2018. Refer to Note 2 to our consolidated financial statements (see Item 8 of this Annual Report on Form 10-K) for more information.

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

The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed here. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in this section as well as factors described in Part I, Item 1A – "Risk Factors" and "Special Note Regarding Forward-Looking Statements" included elsewhere in this Annual Report on Form 10-K.

Overview

Dicerna™ Pharmaceuticals, Inc. ("we," "us," "our," the "Company," or "Dicerna") is a biopharmaceutical company focused on discovery and development of innovative subcutaneously delivered ribonucleic acid ("RNA") interference ("RNAi")-based therapeutics using our GalXC™ RNAi platform for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases. Within these therapeutic areas, we believe our GalXC RNAi platform will allow us to build a broad pipeline of therapeutics with attractive pharmaceutical properties, including a subcutaneous route of administration, infrequent dosing (e.g., dosing that is monthly or quarterly, and potentially even less frequent), high therapeutic index, and specificity to a single target gene. Our key development programs include DCR PHXC for the treatment of primary hyperoxaluria ("PH"), currently in a Phase 1 clinical trial with expected initiation of registration studies in the first quarter of 2019; DCR HBVS for the treatment of chronic hepatitis B virus ("HBV"), currently in a Phase 1 clinical trial; and an undisclosed product candidate against a serious rare liver disease, currently in Clinical Trial Application ("CTA") or Investigational New Drug application ("IND") enabling studies. Dicerna intends to discover, develop, and commercialize novel therapeutics either on its own or in collaboration with pharmaceutical partners. Dicerna has strategic collaborations with Eli Lilly and Company ("Lilly"), Alexion Pharmaceuticals, Inc. (together with its affiliates, "Alexion"), and Boehringer Ingelheim International GmbH ("BI").

All of our GalXC drug discovery and development efforts are based on the therapeutic modality of RNAi, a highly potent and specific mechanism for silencing the activity of a targeted gene. In this naturally occurring biological process, double-stranded RNA molecules induce the enzymatic destruction of the messenger ribonucleic acid ("mRNA") of a target gene that contains sequences that are complementary to one strand of the therapeutic double-stranded RNA molecule. Our approach is to design proprietary double-stranded RNA molecules that have the potential to engage the enzyme Dicer and initiate an RNAi process to silence a specific target gene. Our GalXC RNAi platform utilizes a proprietary structure of double-stranded RNA molecules configured for subcutaneous delivery to the liver. Due to the enzymatic nature of RNAi, a single GalXC molecule incorporated into the RNAi machinery can destroy hundreds or thousands of mRNAs from the targeted gene.

The GalXC RNAi platform supports Dicerna's long-term strategy to retain a full or substantial ownership stake, subject to the evaluation of potential licensing opportunities as they may arise, and to invest internally in diseases with focused patient populations, such as certain rare diseases. We see such diseases as representing opportunities that carry a relatively higher probability of success, with genetically and molecularly defined disease markers, high unmet medical need, a limited number of centers of excellence to facilitate reaching these patients, and the potential for more rapid clinical development programs. For more complex diseases with multiple gene dysfunctions and larger patient populations, we plan to pursue collaborations that can provide the enhanced scale, resources, and commercial infrastructure required to maximize these prospects, such as our existing collaborative research and license agreements.

We view our operations and manage our business as one segment, which is the discovery, research, and development of treatments based on our RNAi technology platform.

Development Programs

In choosing which development programs to internally advance, we apply scientific, clinical, and commercial criteria that we believe allow us to best leverage our GalXC RNAi platform and maximize value. We are focusing our efforts on three priority therapeutic programs that currently have a CTA filed, IND filed, or are in enabling studies in preparation to submit additional regulatory applications that will be necessary to initiate clinical trials. We are also focusing our efforts on a series of potential programs in the clinical candidate selection stage, or for which a provisional clinical candidate has been selected that may be elevated into IND/CTA enabling studies in the future,

either on our own or in collaboration with larger pharmaceutical companies.

Our three priority programs are: DCR PHXC for the treatment of PH; DCR HBVS for the treatment of chronic HBV infection; and a program for an undisclosed rare liver disease. Our potential programs include additional rare disease programs, a program for the treatment of hypercholesterolemia, and multiple programs in various therapeutic areas involving liver function.

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The table below sets forth the state of development of our various GalXC RNAi platform product candidates as of March 4, 2019:

Status of Dicerna Programs

Our current GalXC RNAi platform development programs are as follows:

- Primary Hyperoxaluria. We are developing DCR PHXC for the treatment of all types of PH. PH is a family of rare, inborn errors of metabolism in which the liver produces excessive levels of oxalate, which in turn causes damage to the kidneys and other tissues in the body.

Our Phase 1 clinical trial of DCR-PHXC called PHYOX has completed dosing. In non-clinical models of PH, DCR PHXC reduces oxalate production to near-normal levels, improving the disease condition. PHYOX is a Phase 1 single ascending-dose study of DCR PHXC in healthy volunteers ("HVs") and study participants with PH. The study is divided into two groups:

- Group A is a placebo-controlled, single-blind, single center study which has enrolled 25 HVs.
- Group B is an open-label, multi-center study enrolling up to 18 participants with PH type 1 ("PH1") or PH type 2 ("PH2"). The primary objective of the study is to evaluate the safety and tolerability of single of DCR PHXC in both groups. The secondary objectives are to evaluate the pharmacodynamic effect of single doses of DCR PHXC on biochemical markers, and to characterize the pharmacokinetics of single doses of DCR PHXC in HVs and study participants with PH.

In May 2018, we dosed the first PH study participant with DCR PHXC in the Group B portion of the Phase 1 clinical trial and received notice from the United States ("U.S.") Food and Drug Administration ("FDA") granting Orphan Drug Designation to DCR PHXC for treatment of PH. In August 2018, the European Medicines Agency's Committee for Orphan Medicinal Products ("COMP") designated DCR PHXC as an orphan medicinal product for the treatment of PH in the European Union ("EU"). Currently, we are in the process of submitting requests for additional regulatory clearances necessary to commence clinical trials for our Phase 2 and Phase 3 studies in 2019. Key regulatory interactions with the FDA and EMA, in anticipation of Phase 2 and Phase 3 studies, are also underway.

We have completed the Group A portion of the study in HVs and started the Group B portion of the study. Group B consists of participants with PH1 dosed at 1.5, 3.0, and 6.0-mg/kg, and participants with PH2 at a dose level of 1.5 and 3.0-mg/kg. As of January 17, 2019, we had dosed all 18 participants (15 PH1 participants and three PH2 participants). We reported interim results from the PHYOX trial on September 5, 2018 and presented updated results (as of October 1, 2018) at Kidney Week in San Diego on October 25, 2018.

As of November 2018, three serious adverse events ("SAEs") have occurred in two participants (one subject experienced two discreet SAEs) in the PHYOX trial; none of these SAEs are related to the study intervention. There have been no clinically significant changes in electrocardiography ("ECG"), vital signs, laboratory, or hematology values. The

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investigators have observed in a total of 32 participants dosed (Group A and B together) mild-to-moderate injection site reactions in nine participants (28%), all of which were transient and resolved without intervention within 24 to 72 hours.

As of a data cut on October 1, 2018, investigators reported that a single 3.0-mg/kg dose of DCR PHXC brought urinary oxalate levels into the normal range (defined as 24-hour excretion <0.460 mmol) at one or more post-dose time points in three out of four PH participants dosed at this level, including a mean maximal reduction in 24-hour urinary oxalate of 65% for the cohort, and a single 1.5-mg/kg dose led to near-normalization (defined as 24-hour excretion ≥0.460 to <0.600 mmol) in three out of four PH1 participants dosed at this level and led to a mean maximal reduction in urinary oxalate of 50% in the five PH patients dosed at that level, including one PH2 patient. Additionally, we intend to initiate a multi-dose study, which we hope will serve as a registration trial, in the first quarter of 2019, pending regulatory feedback.

- Chronic Hepatitis B Virus infection. We have declared a GalXC RNAi platform-based product candidate for the treatment of chronic HBV, DCR HBVS, and have initiated a Phase 1 clinical trial. We received CTA approval from the New Zealand Medicines and Medical Devices Safety Authority in November 2018 and ethics approval from the Health and Disability Ethics Committee in December 2018 for a Phase 1 clinical trial in HVs and patients with chronic HBV. The Phase 1 study was initiated in December 2018 and the first participants were dosed on January 24, 2019. We anticipate human POC data to be available in the fourth quarter of 2019. In addition, we filed for regulatory clearance in Australia, Hong Kong, South Korea, and Thailand in December 2018.

Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen ("HBsAg") and sustained HBV deoxyribonucleic acid ("DNA") suppression in patient plasma or blood. DCR HBVS targets HBV mRNA and leads to greater than 99% reduction in circulated HBsAg in mouse models of HBV infection. DCR HBVS is comprised of a single GalXC molecule that targets HBV mRNAs within the HBsAg gene sequence region. In preclinical studies with a standard mouse model of HBV infection, we have found that targeting this region leads to superior HBsAg suppression, both in magnitude and duration of suppression, compared to targeting within the X gene sequence region. We believe that this difference in suppression derives from the role of the X gene product in indirectly regulating viral gene transcription such that the lack of X gene product leads to higher levels of viral gene transcription. Based on our preclinical studies, we hope to determine the potential of DCR HBVS to reduce HBsAg and HBV DNA levels in the blood of HBV patients in a commercially attractive subcutaneous dosing paradigm.

- An undisclosed rare disease involving the liver. We are developing a GalXC-based therapeutic, targeting a liver-expressed gene involved in a serious rare disease. For competitive reasons, we have not yet publicly disclosed the target gene or disease. The disease is a genetic disorder where mutations in the disease gene lead to the production of an abnormal protein. The protein causes progressive liver damage and fibrosis, in some cases leading to cirrhosis and liver failure, and we believe that silencing of the disease gene will prevent production of the abnormal protein and thereby slow or stop progression of the liver fibrosis. We intend to submit regulatory filings in the second quarter of 2019.

- Additional pipeline programs. We have developed a robust portfolio of additional targets and diseases that we plan to pursue either on our own or in collaboration with partners. We have applied our GalXC technology to multiple gene targets across our disease focus areas of rare diseases, chronic liver diseases, and cardiovascular diseases.

Pursuant to our strategy, we are seeking collaborations with larger and/or more experienced pharmaceutical companies to advance our programs in the areas of chronic liver diseases and cardiovascular diseases, as well as select rare diseases that do not fit our criteria for a priority development program. The chronic liver and cardiovascular disease areas represent large and diverse patient populations, requiring complex clinical development and commercialization paths that we believe can be more effectively pursued in collaboration with larger pharmaceutical companies. Certain rare diseases require complex clinical development and commercialization paths aligned with existing treatment paradigms that we believe can be more effectively pursued in collaboration with companies possessing certain rare disease expertise.

For our additional rare disease opportunities, we are continuing to assess their potential for clinical success and market opportunity while optimizing our GalXC molecules. For our additional pipeline programs, we may utilize more advanced versions of our GalXC technology that further improve pharmaceutical properties of the GalXC molecules, including enhancing the duration of action and potency. We have further optimized our GalXC technology platform, enabling the development of next generation GalXC molecules. Improvements to our GalXC compound include modification of the tetraloop end of the molecule, which can be applied to any target gene, resulting in a substantially longer duration of action and higher potency of target gene silencing in animal models across multiple targets. Modification of the tetraloop only impacts the sense strand and does not impact the antisense strand. These modifications are unique to our GalXC molecules and, we believe, provide a competitive advantage for the Company.

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Partner Development Programs

Lilly Collaboration

On October 25, 2018, we entered into a Collaboration and License Agreement with Lilly (the "Lilly Collaboration Agreement"). The Lilly Collaboration Agreement is for the discovery, development, and commercialization of potential new medicines in the areas of cardiometabolic disease, neurodegeneration, and pain. Under the terms of the Lilly Collaboration Agreement, we and Lilly will seek to use our proprietary GalXC RNAi technology platform to progress new drug targets toward clinical development and commercialization. In addition, we will collaborate with Lilly to extend the GalXC RNAi platform technology to non-liver (i.e., non-hepatocyte) tissues, including neural tissues.

The Lilly Collaboration Agreement provides that we will work exclusively with Lilly in the neurodegeneration and pain fields with the exception of mutually agreed upon orphan indications. Additionally, we will work exclusively with Lilly on select targets in the cardiometabolic field. Under the Lilly Collaboration Agreement, we will provide Lilly with exclusive and non-exclusive licenses to support the companies' activities and to enable Lilly to commercialize products derived from or containing compounds developed pursuant to such agreement. The Lilly Collaboration Agreement contemplates in excess of 10 targets.

Under the terms of the Lilly Collaboration Agreement, Lilly paid us a non-refundable, non-creditable upfront payment of $100.0 million, and made a concurrent stated $100.0 million equity investment in us at a premium pursuant to a share issuance agreement between the parties (the "Lilly Share Issuance Agreement"). Under the Lilly Collaboration Agreement, we are also eligible to potentially receive up to approximately $350.0 million per target in development and commercialization milestones, in addition to a $5.0 million payment due for each of the non-hepatocyte targets when a product achieves proof of principle in an animal model. In addition, the Lilly Collaboration Agreement also provides that Lilly will pay us mid-single to low-double digit royalties on product sales on a country-by-country and product-by-product basis until the later of expiration of patent rights in a country, the expiration of data or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement. Through December 31, 2018, no revenue has been recognized associated with the Lilly Collaboration Agreement.

Alexion Collaboration

On October 22, 2018, we and Alexion entered into a Collaborative Research and License Agreement (the "Alexion Collaboration Agreement") for the joint discovery and development of RNAi therapies for complement-mediated diseases. Under the terms of the Alexion Collaboration Agreement, we will collaborate with Alexion on the discovery and development of subcutaneously delivered GalXC candidates, currently in preclinical development, for the treatment of complement-mediated diseases with potential global commercialization by Alexion. We will lead the joint discovery and research efforts through the preclinical stage, and Alexion will lead development efforts beginning with Phase 1 studies. We will be responsible for manufacturing of the GalXC candidates through the completion of Phase 1, the costs of which will be paid by Alexion. Alexion will be solely responsible for the manufacturing of any product candidate subsequent to the completion of Phase 1.

The Alexion Collaboration Agreement also provides Alexion with exclusive worldwide licenses as well as development and commercial rights for two of our preclinical, subcutaneously delivered GalXC RNAi candidates and an exclusive option for the discovery and development of GalXC RNAi candidates against two additional complement pathway targets.

Under the terms of the Alexion Collaboration Agreement, Alexion paid us a non-refundable, non-reimbursable, and non-creditable upfront payment of $22.0 million, with Alexion Pharmaceuticals making a concurrent stated $15.0 million equity investment at a premium in Dicerna pursuant to a share issuance agreement between us and Alexion Pharmaceuticals (the "Alexion Share Issuance Agreement"). The Alexion Collaboration Agreement also provides for potential additional payments to Dicerna of up to $600.0 million from proceeds from target option exercises and development and sales milestones, as defined in the agreement, which is comprised of option exercise fees of up to $20.0 million, representing $10.0 million for each of the candidates selected; development milestones of up to $105.0 million for each product; and aggregate sales milestones of up to $160.0 million. Under the agreement, Alexion will also pay us mid-single to low-double digit royalties on potential product sales on a country-by-country,

product-by-product basis until the later of the expiration of patent rights in a country, the expiration of market or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement. Through December 31, 2018, we recognized $0.1 million in revenue associated with the Alexion Collaboration Agreement.

BI Collaboration

On October 27, 2017, we entered into a Collaborative Research and License Agreement with BI (the “BI Agreement”), pursuant to which we and BI agreed to jointly research and develop product candidates for the treatment of chronic liver diseases, with an initial focus on nonalcoholic steatohepatitis (“NASH”) using our GalXC platform. NASH is caused by the buildup of fat in the liver,

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potentially leading to liver fibrosis and cirrhosis. NASH has an especially high prevalence among obese and diabetic patients and is an area of high unmet medical need.

The BI Agreement is for the development of product candidates against an initial undisclosed target gene and includes an option for BI to add the development of product candidates that target a second gene (the "Additional Target"). We are working exclusively with BI to develop the product candidates against the undisclosed target gene. We are responsible for the discovery and initial profiling of the product candidates, including primary preclinical studies, synthesis, and delivery. BI is responsible for evaluating and selecting the product candidates for further development. If BI selects one or more product candidates, it will be responsible for further preclinical development, clinical development, manufacturing, and commercialization of those products. Also pursuant to the BI Agreement, we granted BI a worldwide license in connection with the research and development of the product candidates and have transferred to BI certain intellectual property rights of the product candidates selected by BI for clinical development and commercialization. We also may provide assistance to BI in order to help BI further develop selected product candidates.

Under the terms of the BI Agreement, BI agreed to pay us a non-refundable upfront payment of $10.0 million for the first target. During the term of the research program, BI will reimburse Dicerna the cost of materials and third-party expenses that have been included in the preclinical studies up to an agreed-upon limit. We are eligible to receive up to $191.0 million in potential development and commercial milestones related to the initial target. We are also eligible to receive royalty payments on potential global net sales, subject to certain adjustments, tiered from high single digits up to low double-digits.

In October 2018, BI exercised its option under the BI Agreement to add the development of product candidates targeting an Additional Target to the development activities governed by the BI Agreement. On December 31, 2018, we entered into an Additional Target Agreement (the "ATA") with BI. The ATA requires the parties to agree on a research work plan and budget for the Additional Target. The ATA also amends the BI Agreement to provide BI with the option to add the development of product candidates targeting a further additional undisclosed gene target to the BI Agreement (the "Second Target" option) for a three-year period, and to provide for the delivery of a replacement product candidate by us to BI in the event that a product candidate under the BI Agreement or the ATA fails at certain stages of preclinical or clinical development.

Under the terms of the ATA, in accordance with the terms of the BI Agreement, BI agreed to pay us a non-refundable upfront payment of $5.0 million to exercise its initial option for development related to the Additional Target. Under the terms of the ATA, during the term of the research program, BI will reimburse us for certain expenses. We are eligible to receive up to $170.0 million in potential development and commercial milestones related to the Additional Target. We are also eligible to receive tiered royalty payments on potential global net sales, subject to certain adjustments, in the mid-single digits. Other than as set forth in the ATA, development of the Additional Target will be subject to the terms of the BI Agreement. Under the ATA, if BI elects to exercise the second option, the parties would agree to a research work plan and budget for the additional gene and negotiate development and commercialization milestones and royalty payments to Dicerna. BI would make another option fee payment to us of $5.0 million.

Through December 31, 2018, we have recognized $7.1 million in revenue associated with the BI Agreement.

Other Development Programs

We have also developed a wholly-owned clinical candidate, DCR BCAT, targeting the ß-catenin oncogene. DCR-BCAT is based on an extended version of our earlier generation non-GalXC RNAi technology and is delivered by our lipid nanoparticle ("LNP") tumor delivery system, EnCore™. We plan to out-license the DCR-BCAT opportunity, given our focus on our GalXC platform-based programs.

Critical Accounting Policies and Significant Judgments and Estimates

Our discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of our consolidated financial statements requires us to make estimates and apply judgments that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the revenue and expenses incurred during the reported periods. We base our estimates on historical experience and on various other factors that we believe are reasonable under the

circumstances. Actual results may differ from these estimates and could have a material impact on our consolidated financial statements.

While our significant accounting policies are more fully described in the notes to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies are the most critical to understanding the judgments and estimates applied in our reported financial results.

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Revenue recognition

We generate revenue from research collaboration and license agreements with third-party customers. Goods and services in the agreements typically include (i) the grant of licenses for the use of our technology and (ii) the provision of services associated with the research and development of customer product candidates. Such agreements may provide for consideration to us in the form of upfront payments, research and development services, option payments, milestone payments, and royalty payments on licensed products.

We account for a contract when we have approval and commitment from both parties, when the rights of the parties are identified, when payment terms are identified, when the contract has commercial substance, and when collectability of consideration is probable.

In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under each of our agreements, management completes the following steps: (i) identifies the contract(s) with a customer; (ii) identifies the performance obligations in the contract; (iii) measures the transaction price, including whether there are any constraints on variable consideration; (iv) allocates the transaction price to the performance obligations; and (v) recognizes revenue when (or as) we satisfy each performance obligation.

In order to account for our contracts with customers, we identify the promised goods or services in the contract and evaluate whether such promised goods or services represent performance obligations. We account for those components as separate performance obligations when the following criteria are met:

- the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer, and
- our promise to transfer the good or service to the customer is separately identifiable from other promises in the contract.

This evaluation requires subjective determinations and requires us to make judgments about the promised goods and services and whether such goods and services are separable from the other aspects of the contractual relationship. In determining the performance obligations, we evaluate certain criteria, including whether the promised good or service is capable of being distinct and whether such good or service is distinct within the context of the contract, based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research, manufacturing, and commercialization capabilities of the partner; the availability of research and manufacturing expertise in the general marketplace; and the level of integration, interrelation, and interdependence among the promises to transfer goods or services.

At contract inception, we determine the standalone selling price for each performance obligation identified in the contract. If an observable price of the promised good or service sold separately is not readily available, we utilize assumptions that require judgment to determine the standalone selling price for each performance obligation identified in the underlying contract, which may include development timelines, probabilities of technical and regulatory success, reimbursement rates for personnel costs, forecasted revenues, potential limitations to the selling price of the product, expected technological life of the product, and discount rates. The transaction price is allocated among the performance obligations using the relative selling price method, and the applicable revenue recognition criteria are applied to each of the separate performance obligations.

Licenses of intellectual property: If a license granted to a customer to use our intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenue from consideration allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, we apply judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, to conclude upon the appropriate method of measuring progress for purposes of recognizing revenue related to consideration allocated to the performance obligation. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

Milestone payments: At the inception of each contract with a customer that includes development or regulatory milestone payments, we evaluate whether the milestones are considered probable of being reached and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant

revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or of the licensee, such as regulatory approvals, are assessed as to the probability of achieving the related milestones. At the end of each subsequent reporting period, we re evaluate the probability of achievement of all milestones and any related constraint, and, if necessary, adjusts the estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis and are recorded as revenue and through earnings in the period of adjustment.

Options: Customer options, such as options granted to allow a licensee to choose to research and develop product candidates against target genes to be identified in the future, generally do not provide a material right to the customer and therefore do not give

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rise to a separate performance obligation. As such, the additional consideration that would result from a customer exercising an option in the future is not included in the transaction price for the current contract. Instead, additional option fee payments are recognized or begin being recognized as revenue when the licensee exercises the options, and the exercise of the option would be treated as a separate contract for accounting purposes.

Research and development services: Arrangements that include a promise to provide research or development services at the licensee's discretion are assessed to determine whether the services provide a material right to the licensee and are capable of being distinct, are not highly interdependent or do not significantly modify one another, and if so, the services are accounted for as separate performance obligations as the services are provided to the customer. Otherwise, when research or development services are determined not to be capable of being distinct or distinct within the context of the contract, those services are combined with the performance obligation that includes the underlying license.

Royalties: For arrangements that include sales-based royalties, including commercial milestone payments based on the achievement of a specified level of sales, and when the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any out-licensing arrangement.

We receive payments from our licensees as established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt or when due and may require deferral of revenue recognition to a future period until (or as) we satisfy our performance obligations under these arrangements. Where applicable, amounts are recorded as contracts receivable when our right to consideration is unconditional. We do not assess whether a contract with a customer has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less.

Stock-based compensation

Our stock-based compensation programs grant awards which may include stock options, restricted common stock, rights to acquire stock, and other stock-based awards. Stock-based compensation cost is measured at the grant date based on the fair value of the award and is recognized as expense over the requisite service period, which generally represents the vesting period, and includes an estimate of the awards that will be forfeited.

We estimate the fair values of stock options granted to our employees and non-employees on the grant date, rights to acquire stock granted under our Employee Stock Purchase Plan, and the resulting stock-based compensation expense, using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of judgment to develop input assumptions, some of which are highly subjective, including: (i) the fair value of our common stock on the date of grant; (ii) the expected volatility of our stock; (iii) the expected term of the award; (iv) the risk-free interest rate; and (v) expected dividends. In applying these assumptions, we consider the following factors:

Fair Value of Common Stock: We use the market closing price for our common stock on the date of grant to determine the fair value of our common stock on the date of grant.

Expected Term: The expected term assumption represents the weighted average period the stock options are expected to be outstanding. We use the simplified method to calculate the expected term for options granted to employees as our stock option grants are considered "plain vanilla" and we do not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term due to the limited period of time our common stock has been publicly traded. The simplified method calculates the expected term as the average time-to-vesting and the contractual life of the options. We plan to continue to use the simplified method until we have sufficient exercise history as a publicly traded company.

Expected Volatility: Due to the lack of company-specific historical and implied volatility data, we base our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The computation of expected volatility is based on the historical volatility using the daily closing prices of a representative group of companies with similar characteristics to us, including stage of life cycle, financial leverage, enterprise value, risk profiles, and position within the industry, along with historical share price information sufficient to meet the expected life of the stock-based awards. We believe the group selected has sufficient similar economic and industry

characteristics and includes companies that are most representative our company. We intend to continue to consistently apply this process using the same or similar public companies until a sufficient amount of historical information regarding the volatility of our own common stock share price becomes available, or unless circumstances change such that the identified companies are no longer similar to us, in which case, more suitable companies whose share prices are publicly available would be utilized in the calculation.

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Risk-Free Interest Rate: The risk-free interest rate is based on the implied yield available on U.S. Treasury zero-coupon issues in effect at the time of grant for periods corresponding with the expected term of the option.
Expected Dividend Yield: We have never paid and have no plans to pay dividends on our common stock. Therefore, we use an expected dividend yield of zero.
Stock-based compensation expense recognized in the financial statements is based on awards that are ultimately expected to vest. Accordingly, we are also required to estimate forfeitures at the time of grant, and to revise those estimates in subsequent periods if actual forfeitures differ from estimates. We use historical data to estimate pre-vesting option forfeitures and record stock-based compensation expense only for those awards that are expected to vest. Our forfeiture rate estimates are based on an analysis of our actual forfeiture experience, employee turnover behavior, and other factors. The impact of any adjustments to our forfeiture rates would be recorded as a cumulative adjustment in the period of adjustment. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised.

Recent Accounting Pronouncements

A summary of recent accounting pronouncements that we have adopted or expect to adopt is included in Note 2 – Summary of Significant Accounting Policies to our consolidated financial statements (see Part I, Item 8 – “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K). Additional information regarding relevant accounting pronouncements is provided below.

Adopted in 2018

Revenue recognition

In May 2014, the accounting guidance related to revenue recognition was amended to provide a single, comprehensive standard for accounting for revenue from contracts with customers. The new guidance became effective for us on January 1, 2018 and applies to all contracts with customers. Under the new guidance, revenue is recognized for contracts with customers based on a model that includes identifying performance obligations and determining and allocating the transaction price to the performance obligations identified in the contract. Revenue is recognized as those performance obligations are satisfied. We applied this new guidance retrospectively to all prior periods presented, and adoption of this new guidance did not have a significant quantitative impact on our consolidated financial statements. However, adoption of this guidance resulted in additional revenue-related disclosures in the notes to our consolidated financial statements.

Not yet adopted

Leases

In February 2016, accounting guidance related to leases was issued that will require an entity to recognize leased assets and the rights and obligations created by those leased assets on the balance sheet and to disclose key information about an entity’s leasing arrangements. This guidance became effective for us on January 1, 2019. We expect that the adoption of this guidance will impact our consolidated financial statements and notes thereto, largely resulting from the recognition of right of use assets and related liabilities related to our non-cancelable operating lease arrangements for office and laboratory spaces. The Company is in the process of determining whether it will utilize the optional transition method presented in the new guidance.

As of December 31, 2018, and as presented in Note 14 – Commitments and Contingencies to our consolidated financial statements (see Part I, Item 8 – “Financial Statements and Supplementary Data” of this Annual Report on Form 10-K), our total future minimum lease obligation associated with our Cambridge, MA lease was $3.3 million, which will remain outstanding at the time we adopt the new guidance.

Recent Developments

Lexington lease

On January 2, 2019, we entered into a non-cancelable real property lease agreement with Hayden Office Trust under a Declaration of Trust dated August 24, 1977, as the same may have been amended, for approximately 80,872 square feet of laboratory and office space in Lexington, Massachusetts (the “Lexington Lease”). We intend to move our corporate headquarters and research facility to this location upon occupancy, which is expected to occur in the fourth quarter of 2019.

The original term (the “Original Term”) of the Lexington Lease is seven years, commencing on the earlier of (a) the date on which the premises are ready for occupancy under the terms of the lease, or (b) the date on which we commence occupancy of any

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portion of the premises for the permitted uses under the lease. We have options to extend the term of the lease for two additional successive periods of five years each (the "Extension Periods").

Annual fixed rent is approximately $3.9 million for the first 12-month period during the Original Term, increasing on an annual basis until reaching approximately $4.7 million for the seventh 12-month period during the Original Term. The Lexington Lease provides for an aggregate fixed rent of approximately $30.1 million during the seven-year Original Term. We will agree upon annual fixed rent during the Extension Periods with the landlord following our provision of notice of intention to exercise an extension option. If we cannot reach an agreement on annual fixed rent during an Extension Period with the landlord, we will have the right to seek, subject to the terms of the Lexington Lease, a broker determination of the prevailing market rent, and the annual fixed rent during such Extension Period will be the prevailing market rent determined by the broker.

In addition to the annual fixed rent, we will be responsible for certain customary operating expenses and real estate taxes specified in the agreement. The Lexington Lease also contains customary default provisions allowing the landlord to terminate the lease or seek damages if we fail to cure certain breaches of our obligations under the lease within specified periods of time. In addition, we will be obligated to indemnify the landlord for certain losses incurred in connection with our use or occupancy of the premises.

Cambridge sublease

On January 4, 2019, we entered into a non-cancelable real property sublease agreement with PPF OFF 150 Cambridge Park Drive, LLC (the "Landlord") and International Business Machines Corporation (the "Sublandlord"), for approximately 9,653 square feet of office space in Cambridge, Massachusetts ("Cambridge Sublease"). The term of the sublease commenced on January 11, 2019, the date that the Landlord provided written consent to the Cambridge Sublease, and extends through the sublease expiration date of July 30, 2021. The Cambridge Sublease provides for an aggregate fixed rent of approximately $0.8 million during the term of the sublease.

Alnylam Settlement Agreement

On April 18, 2018, we entered into a Confidential Settlement Agreement and General Release (the "Settlement Agreement") with Alnylam Pharmaceuticals, Inc. ("Alnylam"), resolving all ongoing litigation between us and Alnylam. The terms of the Settlement Agreement include mutual releases and dismissals with prejudice of all claims and counterclaims in the following litigation between the parties: (i) Alnylam Pharmaceuticals, Inc. v. Dicerna Pharmaceuticals, Inc., No. 15-4126 pending in the Massachusetts Superior Court for Middlesex County and (ii) Dicerna Pharmaceuticals, Inc., v. Alnylam Pharmaceuticals, Inc. No.1:17-cv-11466 pending in the United States District Court for the District of Massachusetts. Under the terms of the Settlement Agreement, in the second quarter of 2018, we paid Alnylam an upfront cash payment of $2.0 million and issued 983,208 shares of Dicerna common stock, which was valued at $10.3 million based on the closing price of our common stock on the date the Settlement Agreement was executed, $2.5 million paid in November 2018, and a final payment of $10.5 million in January 2019. The issuance of shares of our common stock were pursuant to a share issuance agreement between the parties.

Under the Settlement Agreement, for periods ranging from 18 months up to four years, we will be restricted in our development and other activities relating to oligonucleotide-based therapeutics directed toward a defined set of eight Alnylam targets (the "Oligo Restrictions"). The Oligo Restrictions pertain to targets where Dicerna does not have, or does not currently intend to have, a therapeutic program, or are expected to be consistent with our execution on programs in the normal course of business. The Settlement Agreement did not include any admission of liability or wrongdoing by either party or any licenses to any other intellectual property from either party.

As a result of the recently executed partnership agreement with Lilly, we expected that our remaining obligation as of December 31, 2018 would become due. As a result, we recalculated the cash obligation to an estimated present value of $10.5 million based on the expected timing of the remaining payments and recorded it in current liabilities as of December 31, 2018.

Financial Operations Overview

Executive Overview

Our results of operations and liquidity and capital resources for and as of the year ended December 31, 2018, compared to the prior year, reflect the following:

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In April 2018, we entered into the Settlement Agreement with Alnylam, resolving all ongoing litigation between the two companies. Pursuant to the terms of the Settlement Agreement, Dicerna paid to Alnylam an upfront payment of $2.0 million, agreed to a future payment of $13.0 million (of which $2.5 million was paid in November 2018 and $10.5 million was paid in January 2019), and issued 983,208 shares of Dicerna common stock worth $10.3 million to Alnylam.

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- On September 6, 2018, we entered into an underwriting agreement with Citigroup Global Markets Inc. and Leerink Partners LLC as representatives of the underwriters relating to the underwritten public offering of 7,680,492 shares of our common stock, and the grant to the underwriters of a 30-day option to purchase up to an additional 1,152,073 shares of our common stock. We completed the sale of 8,832,565 shares to the underwriters on September 11, 2018, which resulted in net proceeds to us of approximately $107.7 million, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.
- In October 2018, we entered into the Alexion Collaboration Agreement for the joint discovery and development of RNAi therapies for complement-mediated diseases. Under the terms of the agreement, we received an upfront payment of $22.0 million, with Alexion making a concurrent stated $15.0 million equity investment at a premium in the Company.
- In October 2018, we entered into the Lilly Collaboration Agreement for the discovery, development, and commercialization of potential new medicines in the areas of cardiometabolic disease, neurodegeneration, and pain. Under the terms of the agreement, we received an upfront payment of $100.0 million in January 2019, and, in December 2018, Lilly made a stated $100.0 million equity investment at a premium in the Company.
- In October 2018, BI exercised its Second Target option, which entitled the Company to a non-refundable payment of $5.0 million upon the agreement of a research work plan and budget, which were finalized in January 2019. The terms of the Second Target option exercise and related rights and obligations associated with the Second Target were agreed upon through an Additional Target Agreement ("ATA") that was executed with BI on December 31, 2018. Since the Second Target option was not considered to be a material right, the exercise of the Second Target option is being treated as a separate contract for accounting purposes.

Revenue from collaborative arrangements

Our revenue from collaboration arrangements to date has been generated primarily through research funding, license fees, option exercise fees, and preclinical development payments under our research collaboration arrangements with Lilly, Alexion, and BI. We have not generated any commercial product revenue, nor do we expect to generate any product revenue for the foreseeable future.

In the future, we may generate revenue from a combination of research and development payments, license fees and other upfront payments, milestone payments, product sales, and royalties in connection with our current or future collaborations with partners. We expect that any revenue we generate will fluctuate in future periods as a result of the timing of our or our collaborators' achievement of preclinical, clinical, regulatory, and commercialization milestones, to the extent achieved, the timing and amount of any payments to us relating to such milestones, and the extent to which any of our product candidates are approved and successfully commercialized by us or a collaborator. If we, our current collaboration partners, or any future collaborator fails to develop product candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenue from collaboration arrangements, and our results of operations and financial position, would be materially and adversely affected.

Lilly Collaboration and License Agreement

On October 25, 2018, we entered into the Lilly Collaboration Agreement, pursuant to which we were entitled to receive a non-refundable, non-creditable upfront payment of $100.0 million and a concurrent stated $100.0 million equity investment at a premium pursuant to a share issuance agreement between the parties (the "Lilly Share Issuance Agreement"). At December 31, 2018, the non-refundable, non-creditable upfront payment of $100.0 million was recorded as a contract receivable in the accompanying consolidated balance sheets. During the year ended December 31, 2018, we did not recognize any revenue associated with the Lilly Collaboration Agreement.

Under the Lilly Collaboration Agreement, we are also eligible to potentially receive up to approximately $350.0 million per target in development and commercialization milestones, in addition to a $5.0 million payment due for each of the non-hepatocyte targets when a product achieves proof of principle in an animal model. In addition, the Lilly Collaboration Agreement also provides that Lilly will pay us mid-single to low-double digit royalties on product sales on a country-by-country and product-by-product basis until the later of expiration of patent rights in a country, the expiration of data or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement. Lilly may terminate the Lilly Collaboration Agreement at any time without cause following a 90-day notice period.

Alexion Collaborative Research and License Agreement

On October 22, 2018, we entered into the Alexion Collaboration Agreement, pursuant to which we received a non-refundable, non-reimbursable, and non-creditable upfront payment of $22.0 million and a concurrent stated $15.0 million equity investment at a premium pursuant to a share issuance agreement between the parties (the "Alexion Share Issuance Agreement"). Alexion made both

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payments during the fourth quarter of 2018. During the year ended December 31, 2018, we recognized $0.1 million in revenue associated with the Alexion Collaboration Agreement.

The Alexion Collaboration Agreement also provides for potential additional payments of up to $600.0 million, from proceeds from target option exercises and development and sales milestones, as defined in the agreement, which is comprised of option exercise fees of up to $20.0 million, representing $10.0 million for each of the candidates selected; development milestones of up to $105.0 million for each product; and aggregate sales milestones of up to $160.0 million. Under the agreement, Alexion will also pay us mid-single to low-double digit royalties on potential product sales on a country-by-country, product-by-product basis until the later of the expiration of patent rights in a country, the expiration of market or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement.

BI Agreements

On October 27, 2017, we entered into the BI Agreement, as discussed above, pursuant to which we were entitled to receive a non-refundable upfront payment of $10.0 million, less a refundable withholding tax in Germany of $1.6 million. BI paid us $8.4 million during the fourth quarter of 2017. The non-refundable upfront payment was subject to a German withholding tax, which was withheld by BI and remitted to the German tax authority in accordance with local tax law. We collected the remaining $1.6 million during 2018. During the years ended December 31, 2018 and 2017, we recognized $6.1 million and $1.0 million, respectively, in revenue associated with the BI Agreement, including reimbursable third-party research expenses that are billable to BI.

The deliverables at the effective date of the BI Agreement include delivery of intellectual property, conducting agreed-upon research program services, and providing BI the exclusive option right to reserve additional targets. We concluded that the performance of additional research for any additional target, if the underlying target option is exercised by BI, is not a deliverable of the agreement at inception because it is a substantive option and is not priced at a significant and incremental discount. Milestone payments that are contingent upon our performance under the BI Agreement include developmental milestones, as defined in the agreement, totaling $99.0 million. We view these milestones as substantive and have excluded the amounts from allocable consideration at the outset of the arrangement. All potential commercial milestones, as defined in the agreement, totaling $95.0 million, will be accounted for in the same manner as royalties and recorded as revenue upon achievement of the applicable milestone, assuming all other revenue recognition criteria are met.

In October 2018, BI exercised their option under the BI Agreement to add the development of product candidates targeting an additional gene (the “Additional Target”) to the development activities governed by the BI Agreement. On December 31, 2018, we entered into an Additional Target Agreement (the “ATA”) with BI. The ATA requires the parties to agree on a research work plan and budget for the Additional Target, which was completed in January 2019, at which point BI paid us a $5.0 million option exercise fee. The ATA also amends the BI Agreement to provide BI with the option to add the development of product candidates targeting a further additional undisclosed gene for a three-year period, and to provide for the delivery of a replacement product candidate by us to BI in the event that a product candidate under the BI Agreement or the ATA fails at certain stages of preclinical or clinical development. Under the ATA, during the term of the research program, BI will reimburse us for certain expenses. We are eligible to receive up to $170.0 million in potential development and commercial milestones related to the Additional Target. We are also eligible to receive tiered royalty payments on potential global net sales, subject to certain adjustments, in the mid-single digits. Other than as set forth in the ATA, development of the Additional Target will be subject to the terms of the BI Agreement. Under the ATA, if BI elects, in their sole discretion, to exercise their option to add the development of product candidates targeting a further additional undisclosed gene, the parties would agree to a research work plan and budget for the additional gene and negotiate development and commercialization milestones and royalty payments to us. BI would make another option fee payment to us of $5.0 million.

Research and development expenses

Research and development expenses consist of costs associated with our research activities, including discovery and development of our GalXC molecules and drug delivery technologies, clinical and preclinical development activities, and research activities under our research collaboration and license agreements. Our research and development expenses include:

- direct research and development expenses incurred under arrangements with third parties, such as contract research organizations, contract manufacturing organizations, and consultants;
- platform-related lab expenses, including lab supplies, license fees, and consultants;
- employee-related expenses, including salaries, benefits, and stock-based compensation expense; and
- facilities, depreciation, and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

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We expense research and development costs as they are incurred. We account for non-refundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received. A significant portion of our research and development costs are not tracked by project as they benefit multiple projects or our technology platform.

The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time-consuming. We or any of our current or future collaborators may never succeed in obtaining marketing approval for any of our product candidates. The probability of success for each product candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability, and commercial viability. All of our research and development programs are at an early stage, and successful development of future product candidates from these programs 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 product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to our ability to maintain or enter into collaborations with respect to each product candidate, the scientific and clinical success of each product candidate, as well as ongoing assessments as to the commercial potential of product candidates. We will need to raise additional capital and may seek additional collaborations in the future in order to advance our various product candidates. Additional private or public financings may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related benefits, including stock-based compensation, related to our executive, finance, legal, business development, and support functions. Other general and administrative expenses include travel expenses, professional fees for legal (excluding settlement and litigation expenses related to the Alnylam Settlement), audit, tax, and other professional services, and allocated facility-related costs not otherwise included in research and development expenses.

Litigation expense

Litigation expense consists of legal fees and expenses solely related to the litigation with Alnylam and the Settlement Agreement.

Interest income

Interest income consists of interest income earned on our cash and cash equivalents, held-to-maturity investments, and restricted cash equivalents.

Interest expense

Interest expense represents the accretion of the net present value of the litigation settlement payable to its carrying value.

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Results of Operations

Comparison of the years ended December 31, 2018 and 2017

The following table summarizes the results of our operations for the periods indicated (amounts in thousands, except percentages):

	YEAR ENDED DECEMBER 31,			
	2018	2017	$ CHANGE	% CHANGE
Revenue from collaborative arrangements	$6,176	$1,030	$ 5,146	499.6 %
Operating expenses:				
Research and development	45,711	35,888	9,823	27.4 %
General and administrative	21,685	16,838	4,847	28.8 %
Litigation expense	29,132	9,043	20,089	222.1 %
Total operating expenses	96,528	61,769	34,759	56.3 %
Loss from operations	(90,352)	(60,739)	(29,613)	48.8 %
Other income (expense):				
Interest income	2,102	539	1,563	290.0 %
Interest expense	(603)	—	(603)	— %
Total other income, net	1,499	539	960	178.1 %
Net loss	(88,853)	(60,200)	(28,653)	47.6 %
Dividends on redeemable convertible preferred stock	—	(10,111)	10,111	(100.0)%
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	—	(6,144)	6,144	(100.0)%
Deemed dividend on conversion of redeemable convertible preferred stock	—	(3,837)	3,837	(100.0)%
Net loss attributable to common stockholders	$(88,853)	$(80,292)	$ (8,561)	10.7 %

Revenue from collaborative arrangements

During the year ended December 31, 2018, revenue from collaborative arrangements increased $5.1 million compared to 2017 due to the recognition of a full year of revenue under the BI Agreement and our entry into the Alexion Collaboration Agreement.

Revenue recognized under the BI Agreement during the years ended December 31, 2018 and 2017 was $6.1 million and $1.0 million, respectively. This revenue represents the periodic amortization of a non-refundable upfront payment of $10.0 million and $0.3 million of certain reimbursable costs pursuant to the BI Agreement, which was signed in the fourth quarter of 2017. As of December 31, 2018, we expect to recognize the remaining $3.2 million of the non-refundable upfront payment on a straight-line basis through August 2019.

Revenue recognized under the Alexion Collaboration Agreement during the year ended December 31, 2018 was $0.1 million. At December 31, 2018, the $31.3 million of consideration received and allocated to the revenue element of the arrangement relates to our partially unsatisfied performance obligations and is recorded as a contract liability presented in deferred revenue, of which $11.7 million was included in the current portion of deferred revenue. As of December 31, 2018, we expect to recognize this amount over the remaining research program term, which we estimate will extend through the fourth quarter of 2023.

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Research and development expenses

The following table summarizes our research and development expenses incurred during the periods indicated (amounts in thousands, except percentages):

	YEAR ENDED DECEMBER 31,			
	2018	2017	$ CHANGE	% CHANGE
Direct research and development expenses	$22,912	$15,898	$ 7,014	44.1 %
Platform-related expenses	6,325	6,611	(286)	(4.3)%
Employee-related expenses	13,130	10,155	2,975	29.3 %
Facilities, depreciation, and other expenses	3,344	3,224	120	3.7 %
Total	$45,711	$35,888	$ 9,823	27.4 %

Research and development expenses increased for the year ended December 31, 2018 compared to the year ended December 31, 2017 primarily due to direct research and development expenses. The $7.0 million increase in direct research and development expenses is primarily due to increases in clinical development spending of $5.2 million for DCR-PHXC and $1.1 million for DCR-HBVS. In addition, employee-related expenses increased $3.0 million during the year ended December 31, 2018 as a result of increased headcount necessary to support our growth.

Research and development expenses for the years ended December 31, 2018 and 2017 were additionally offset by $0.7 million and $1.1 million of grant income, respectively.

We expect our overall research and development expenses to increase in 2019 and for the foreseeable future, primarily as we complete clinical manufacturing activities, advance preclinical toxicology studies, continue clinical activities associated with our lead product candidates, and as our development efforts continue to increase related to progress made in connection with our collaboration agreements.

General and administrative expenses

General and administrative expenses were $21.7 million and $16.8 million for the years ended December 31, 2018 and 2017, respectively. The increase of $4.8 million is primarily due to increases of $1.9 million in consulting costs, $1.0 million in compensation for our board of directors, and $0.8 million in salary and benefits expense. Our use of consultants increased largely due to business development consulting services and accounting support for the implementation of new accounting standards and preparation for our planned compliance with Sarbanes-Oxley Section 404(b) in 2019, as well as to support new product initiatives. The increase in board of directors' compensation is largely related to stock-based compensation. Salaries and benefits expenses increased as a result of increased headcount required to support our growth.

We expect general and administrative expenses to continue to increase in 2019, largely due to planned investment in staffing, preparation of our new company headquarters, and market readiness activities.

Litigation expense

Litigation expenses are comprised solely of litigation and settlement expenses associated with the litigation with Alnylam. Litigation expenses increased predominantly due to $24.7 million of settlement expenses recorded related to the Settlement Agreement during the year ended December 31, 2018.

Interest income

Interest income is comprised primarily of interest earned from our money market accounts and held-to-maturity investments. Interest income was $2.1 million and $0.5 million for the years ended December 31, 2018 and 2017, respectively. The increase was primarily due to higher held-to-maturity investments balances amounts during the year ended December 31, 2018 primarily resulting from our follow-on public offering in September 2018 and funds received from the collaboration agreements with Lilly and Alexion in the fourth quarter of 2018.

Interest expense

Interest expense of $0.6 million during the year ended December 31, 2018 represents interest expense incurred on our litigation settlement payable.

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Dividends

There were no dividends recorded related to redeemable convertible preferred stock for the year ended December 31, 2018, as all shares of the redeemable convertible preferred stock were converted into shares of our common stock on December 18, 2017.

Net loss attributable to common stockholders

Net loss attributable to common stockholders was $88.9 million and $80.3 million for the years ended December 31, 2018 and 2017, respectively. The overall increase in net loss attributable to common stockholders was due to the increase in net loss from the prior year of $28.7 million, which was partially offset by $10.1 million of dividends and deemed dividends on redeemable convertible preferred shares, as well as $10.0 million of deemed dividends related to the beneficial conversion feature ("BCF") and conversion of redeemable convertible preferred shares in 2017.

Comparison of the years ended December 31, 2017 and 2016

The following table summarizes the results of our operations for the periods indicated (amounts in thousands, except percentages):

	YEAR ENDED DECEMBER 31,			
	2017	2016	$ CHANGE	% CHANGE
Revenue from collaborative arrangements	$1,030	$—	$1,030	— %
Operating expenses:				
Research and development	35,888	41,399	(5,511)	(13.3)%
General and administrative	16,838	15,433	1,405	9.1 %
Litigation expense	9,043	2,916	6,127	210.1 %
Total operating expenses	61,769	59,748	2,021	3.4 %
Loss from operations	(60,739)	(59,748)	(991)	1.7 %
Other income (expense):				
Interest income	539	235	304	129.4 %
Total other income, net	539	235	304	129.4 %
Net loss	(60,200)	(59,513)	(687)	1.2 %
Dividends on redeemable convertible preferred stock	(10,111)	—	(10,111)	— %
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	(6,144)	—	(6,144)	— %
Deemed dividend on conversion of redeemable convertible preferred stock	(3,837)	—	(3,837)	— %
Net loss attributable to common stockholders	$(80,292)	$(59,513)	$(20,779)	34.9 %

Revenue from collaborative arrangements

During the year ended December 31, 2017, we recognized $1.0 million of revenue associated with the BI Agreement. This amount represents partial amortization of the $10.0 million upfront payment received from BI for the first target and $0.3 million in reimbursable costs of certain materials and third-party expenses that have been included in preclinical studies pursuant to the BI Agreement, which was signed in the fourth quarter of 2017. During the year ended December 31, 2017, the upfront payment and reimbursable costs related to the BI Agreement was being recognized ratably over a period of 20 months, which represented our then current estimate for the research term over which the research and development services were to be provided, as well as reimbursable third-party research expenses billable to BI.

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Research and development expenses

The following table summarizes our research and development expenses incurred for the periods indicated (amounts in thousands, except percentages):

	YEAR ENDED DECEMBER 31,			
	2017	2016	$ CHANGE	% CHANGE
Direct research and development expenses	$15,898	$13,603	$ 2,295	16.9 %
Platform-related expenses	6,611	11,302	(4,691)	(41.5)%
Employee-related expenses	10,155	12,972	(2,817)	(21.7)%
Facilities, depreciation, and other expenses	3,224	3,522	(298)	(8.5)%
Total	$35,888	$41,399	$ (5,511)	(13.3)%

Total research and development expenses decreased by $5.5 million during 2017, compared to 2016, despite an overall increase in direct research and development expenses of $2.3 million, which was due to increased drug substance, toxicology study and manufacturing activities associated with our GalXC platform product candidates. The increase was partially offset by a decrease in comparative clinical activities related to our non-GalXC platform clinical trials, which were discontinued during 2016. Platform-related expenses decreased primarily as a result of lower spending in discovery and early development programs, which advanced in 2017 into manufacturing and clinical testing. Employee-related expenses decreased due to an overall decrease in headcount from 2016, along with a decrease in non-cash stock-based compensation costs.

Total research and development expenses for the years ended December 31, 2017 and 2016 were additionally offset by $1.1 million and $0.3 million of grant income, respectively.

General and administrative expenses

General and administrative expenses were $16.8 million and $15.4 million for the years ended December 31, 2017 and 2016, respectively. The increase of $1.4 million was primarily due to higher salaries, benefits, and other employee-related expenses.

Litigation expenses

Litigation expenses are entirely associated with the litigation with Alnylam. Litigation expenses increased $6.1 million due to higher legal fees associated with the litigation with Alnylam during the year ended December 31, 2017.

Interest income

Interest income is comprised primarily of interest earned from our money market accounts and held-to-maturity investments. Interest income was $0.5 million and $0.2 million for the years ended December 31, 2017 and 2016, respectively. The increase was primarily due to higher invested amounts in 2017 primarily as a result of the receipt of net proceeds from the Private Placement, which closed in April 2017.

Dividends

Non-cash dividends of $10.1 million recorded during the year ended December 31, 2017 represent the fair value of accrued dividends on redeemable convertible preferred shares issued to the preferred holders, as well as full accretion of share issuance costs. The fair value of the dividends on the dividend dates of June 30, 2017 and September 30, 2017 was determined using a binary lattice model that captured the intrinsic value of the underlying common stock on the declaration date and the option value of the shares and future dividends. Inputs to the lattice model included an adjusted risk rate, our common stock volatility, the underlying common stock price on the dividend date and management's judgment associated with probability simulations of various outcomes. Dividends were valued at each dividend declaration date based on various inputs and assumptions at that time.

The non-cash deemed dividend related to the BCF of the redeemable convertible preferred stock of $6.1 million for the year ended December 31, 2017 represents the value of a BCF which was recorded on the redeemable convertible preferred shares. The BCF was recognized separately at issuance by allocating a portion of the proceeds equal to the intrinsic value of that feature to additional paid-in capital. The BCF was calculated at the commitment date, which management determined to be the date of issuance. Intrinsic value was calculated as the difference between the

effective conversion price and the fair value of our common stock, multiplied by the number of shares into which the issued shares of redeemable convertible preferred were convertible. The BCF which

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was accreted in full at issuance due to the fact that the underlying shares of redeemable convertible preferred were immediately convertible, and such accretion was recorded as a deemed dividend.

The non-cash deemed dividend on conversion of redeemable convertible preferred stock of $3.8 million for the year ended December 31, 2017 represents the excess fair value of common stock transferred in the conversion transaction to the Preferred Holders over the fair value of common stock issuable pursuant to the original conversion terms. This excess was recorded as a deemed dividend on conversion of the redeemable convertible preferred stock and has been added to net loss to arrive at net loss attributable to common stockholders in our consolidated statement of operations for the year ended December 31, 2017.

As noted above, all shares of redeemable convertible preferred stock were converted into shares of our common stock on December 18, 2017, and, as such, no additional dividends or deemed dividends will be recorded on the redeemable convertible preferred stock in the future. No common stock dividends were recorded during the years ended December 31, 2017 or 2016.

Net loss attributable to common stockholders

Net loss attributable to common stockholders was $80.3 million and $59.5 million for the years ended December 31, 2017 and 2016, respectively. The overall increase in net loss attributable to common stockholders was due to the recording of dividends in 2017 on the redeemable convertible preferred stock, as well as to the deemed dividends related to the BCF and upon conversion of the redeemable convertible preferred stock, and to higher general and administrative expenses, partially offset by higher collaboration and grant revenues and lower research and development expenses.

Liquidity and Capital Resources

Overview

We have historically funded our operations primarily through the public offering and private placement of our securities and consideration received from our collaborative arrangements with Lilly, Alexion, and BI. As of December 31, 2018, we had cash, cash equivalents, and held-to-maturity investments of $302.6 million and $0.7 million in cash equivalents held in restriction. In early 2019, we received an additional $105.0 million in proceeds from the Lilly and BI collaborations, which were offset by the $10.5 million final settlement payment to Alnylam, which became payable upon receipt of the proceeds from Lilly.

On October 31, 2016, a universal shelf registration statement on Form S-3 permitting the sale of up to $150.0 million of our common stock and other securities was declared effective by the U.S. Securities and Exchange Commission (“SEC”). On April 11, 2017, pursuant to an agreement with seven institutional investors (the “Preferred Holders”) we issued and sold 700,000 shares of our newly designated redeemable convertible preferred stock to the Preferred Holders in a private placement for aggregate gross proceeds of $70.0 million, less issuance costs of approximately $0.8 million (the “Private Placement”). On December 18, 2017, we completed the conversion of the redeemable convertible preferred stock and issued an aggregate of 24,206,663 shares of our common stock pursuant to this registration statement, after which no shares of redeemable convertible preferred stock remained outstanding.

On December 18, 2017, we completed an underwritten follow-on public offering and sale of 6,571,428 shares which resulted in the receipt of aggregate gross proceeds of $46.0 million, less underwriter commissions and additional offering expenses totaling approximately $3.2 million.

On May 31, 2018, a universal shelf registration statement on Form S-3 permitting the sale of up to $250.0 million of our common stock and other securities was declared effective by the SEC. In September 2018, we sold an aggregate of 8,832,565 shares of our common stock for gross proceeds of $115.0 million pursuant to this registration statement. We intend to use the net proceeds from the offering for preclinical studies and clinical trials, and to use the remainder of any net proceeds for continued technology platform development, working capital, and general corporate purposes.

In connection with the Alexion Collaboration Agreement, we entered into the Alexion Share Issuance Agreement on October 22, 2018, pursuant to which we agreed to issue to Alexion 835,834 shares of our common stock at a purchase price of $17.95 per share for an aggregate purchase price of approximately $15.0 million.

In connection with the Lilly Collaboration Agreement, we entered into the Lilly Share Issuance Agreement on October 25, 2018, pursuant to which we agreed to issue to Lilly 5,414,185 shares of our common stock at a purchase price of $18.47 per share for an aggregate purchase price of approximately $100.0 million. The shares were issued and

the purchase price was received on December 19, 2018.

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Cash flows

The following table shows a summary of our consolidated cash flows for the periods indicated (amounts in thousands):

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
Net cash provided by (used in) operating activities	$18,298	$(45,327)	$(48,747)
Net cash (used in) provided by investing activities	$(202,731)	$(19,852)	$13,020
Net cash provided by financing activities	$169,883	$112,731	$534

Operating activities

Net cash provided by (used in) operating activities was $18.3 million and $45.3 million for the years ended December 31, 2018 and 2017, respectively. The $63.6 million net increase in cash provided by operating activities was primarily due to an increase of $164.6 million in deferred revenue due to consideration received in connection with the Lilly and Alexion collaboration agreements. This amount was partially offset by a $100.0 million increase in contract receivables associated with the upfront payment for the Lilly collaboration agreement.

Net cash used in operating activities was $45.3 million and $48.7 million for the years ended December 31, 2017 and 2016, respectively. The $3.4 million net decrease in cash used in operating activities is due primarily to the receipt of proceeds, net of the refundable German withholding tax, from BI, as discussed above, and lower research and development expenses, partially offset by higher general and administrative expenses and other net working capital fluctuations.

Investing activities

Net cash used in investing activities for the year ended December 31, 2018 was $202.7 million, compared to net cash used in investing activities of $19.9 million for the year ended December 31, 2017. The increase of $182.9 million in net cash used in investing activities during 2018 primarily relates to an increase of $193.7 million in purchases of held-to-maturity investments as a result of cash received from our follow-on public offering in September 2018, as well as the collaboration agreements signed with Lilly and Alexion in October 2018. This increase was partially offset by an $11.0 million increase in the maturities of held-to-maturity securities.

Net cash used in investing activities for the year ended December 31, 2017 was $19.9 million, compared to net cash provided by investing activities of $13.0 million for the year ended December 31, 2016. The increase of $32.9 million in net cash used in investing activities during 2017 relates primarily to higher purchases of held-to-maturity investments, largely following the receipt of net proceeds from the issuance of the redeemable convertible preferred stock, partially offset by higher maturities of held-to-maturity investments.

Financing activities

Net cash provided by financing activities was $169.9 million and $112.7 million for the years ended December 31, 2018 and 2017, respectively. The increase in cash provided by financing activities of $57.2 million was primarily due to receipt of $124.6 million in proceeds from the issuance of common stock net of underwriters' commissions associated with our follow-on public offering in September 2018 and from the share issuance agreements with Lilly and Alexion during the fourth quarter of 2018. This amount was partially offset by $69.3 million in proceeds from the redeemable convertible preferred stock financing in 2017.

Net cash provided by financing activities was $112.7 million and $0.5 million for the years ended December 31, 2017 and 2016, respectively. The increase of $112.2 million was principally due to the receipt of $69.3 million in net proceeds from the private placement of the redeemable convertible preferred stock, as well as to the receipt of $43.2 million in net proceeds from the 2017 follow-on offering, partially offset by lower proceeds received in connection with stock option exercises and with common stock issuances under our employee stock purchase plan.

Funding requirements

We expect that our primary uses of capital will continue to be third-party clinical research and development services and manufacturing costs, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses, and general overhead costs. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates and the extent to which we may enter into additional

collaborations with third parties to participate in their development and commercialization, we are unable to estimate the amounts of capital outlays and operating expenditures associated

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with our anticipated development activities. However, based on our current operating plan, we believe that available cash, cash equivalents, and held-to-maturity investments will be sufficient to fund the execution of our current clinical and operating plans beyond the year ending December 31, 2020. We have based this estimate on assumptions that may prove to be incorrect, and we could utilize our available capital resources sooner than we currently expect.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially as a result of a number of factors. Our future capital requirements are difficult to forecast and will depend on many factors, including:

- the potential receipt of any milestone payments under the Lilly Collaboration Agreement, the Alexion Collaboration Agreement, or the BI Agreement;
- the terms and timing of any other collaboration, licensing and other arrangements that we may establish;
- the initiation, progress, timing and completion of preclinical studies and clinical trials for our potential product candidates;
- the number and characteristics of product candidates that we pursue;
- the outcome, timing and cost of regulatory approvals;
- delays that may be caused by changing regulatory requirements;
- the cost and timing of hiring new employees to support our continued growth;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
- the costs of filing and prosecuting intellectual property rights and enforcing and defending any intellectual property-related claims;
- the costs of responding to and defending ourselves against complaints and potential litigation;
- the costs and timing of procuring clinical and commercial supplies for our product candidates;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the extent to which we acquire or invest in other businesses, product candidates or technologies.

Until such time, if ever, that we generate product revenue, we expect to finance our future cash needs through a combination of public or private equity offerings, debt financings and research collaboration and license agreements. We may be unable to raise capital or enter into such other arrangements when needed or on favorable terms, or at all. Our failure to raise capital or enter into such other arrangements in a reasonable timeframe would have a negative impact on our financial condition, and we may have to delay, reduce or terminate our research and development programs, preclinical or clinical trials, limit strategic opportunities or undergo reductions in our workforce or other corporate restructuring activities.

Please see the risk factors set forth in Part I, Item 1A – “Risk Factors” in this Annual Report on Form 10-K for additional risks associated with our substantial capital requirements.

Contractual Obligations and Commitments

The following is a summary of our significant contractual obligations as of December 31, 2018 (amounts in thousands):

	Payments Due By Period				
	Total	Less Than 1 Year	More Than 1 Year and Less Than 3 Years	More Than 3 Years and Less Than 5 Years	More Than 5 Years
Operating lease obligation*	$3,259	$ 1,678	$ 1,581	$ —	$ —

* Represents future minimum lease payments under a non-cancelable operating lease for our office and laboratory space in Cambridge, Massachusetts. The end of the lease term is November 30, 2020.

We also have obligations to make future payments to licensors that become due and payable on the achievement of certain development, regulatory, and commercial milestones. We have not included any such potential obligations on our consolidated balance sheet or in the table above, since the achievement and timing of these milestones were not probable or estimable as of December 31, 2018.

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We had a balance of $10.5 million for the litigation settlement liability related to Alnylam recorded on our consolidated balance sheet as of December 31, 2018, which was paid in full in January 2019 upon receipt of the $100.0 million upfront payment from the Lilly collaboration.

Off-Balance Sheet Arrangements

As of December 31, 2018, we did not have any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as “special purpose” entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. Other than the operating lease for our Company headquarters in Cambridge, Massachusetts, we do not engage in off-balance sheet arrangements. Upon adoption of the new lease accounting standard on January 1, 2019, we anticipate that the requirement to capitalize all long-term leases will result in our existing lease, along with any new facility leases, being recorded on our consolidated balance sheet.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

The primary objectives of our investment activities are to ensure liquidity and to preserve principal, while at the same time maximizing the income we receive from our marketable securities without significantly increasing risk. Some of the securities that we invest in may have market risk related to changes in interest rates. As of December 31, 2018, we had cash, cash equivalents, and held-to-maturity investments of $302.6 million. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash and cash equivalents and held-to-maturity investments and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash and cash equivalents or held-to-maturity investments. To minimize the risk in the future, we intend to maintain our portfolio of cash and cash equivalents and held-to-maturity investments in a variety of securities, including commercial paper, money market funds, and government securities.

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ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

DICERNA PHARMACEUTICALS, INC.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of Dicerna Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Dicerna Pharmaceuticals, Inc. and subsidiaries (the "Company") as of December 31, 2018 and 2017, the related consolidated statements of operations, changes in redeemable convertible preferred stock and stockholders' equity, and cash flows, for each of the three years in the period ended December 31, 2018, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2018, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Boston, Massachusetts

March 13, 2019

We have served as the Company's auditor since 2008.

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DICERNA PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share data and par value)

	DECEMBER 31,	
	2018	2017
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$54,239	$68,789
Held-to-maturity investments	248,387	44,889
Contract receivables	100,000	—
Withholding tax receivable	—	1,583
Prepaid expenses and other current assets	2,888	3,415
Total current assets	405,514	118,676
NONCURRENT ASSETS:		
Property and equipment, net	2,718	1,512
Restricted cash equivalents	744	744
Other noncurrent assets	65	70
Total noncurrent assets	3,527	2,326
TOTAL ASSETS	$409,041	$121,002
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$5,013	$4,920
Accrued expenses and other current liabilities	9,649	5,726
Litigation settlement payable	10,500	—
Current portion of deferred revenue	68,893	6,180
Total current liabilities	94,055	16,826
NONCURRENT LIABILITIES:		
Deferred revenue, net of current portion	114,293	3,090
Total noncurrent liabilities	114,293	3,090
TOTAL LIABILITIES	208,348	19,916
COMMITMENTS AND CONTINGENCIES (NOTE 14)		
STOCKHOLDERS' EQUITY:		
Preferred stock, $0.0001 par value – 5,000,000 shares authorized; no shares issued or outstanding at December 31, 2018 or 2017	—	—
Common stock, $0.0001 par value – 150,000,000 shares authorized; 68,210,742 and 51,644,841 shares issued and outstanding at December 31, 2018 and 2017, respectively	7	5
Additional paid-in capital	605,495	417,037
Accumulated deficit	(404,809)	(315,956)
Total stockholders' equity	200,693	101,086
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$409,041	$121,002

The accompanying notes are an integral part of these consolidated financial statements.

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DICERNA PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share data)

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
Revenue from collaborative arrangements	$6,176	$1,030	$—
Operating expenses:			
Research and development	45,711	35,888	41,399
General and administrative	21,685	16,838	15,433
Litigation expense	29,132	9,043	2,916
Total operating expenses	96,528	61,769	59,748
Loss from operations	(90,352)	(60,739)	(59,748)
Other income (expense):			
Interest income	2,102	539	235
Interest expense	(603)	—	—
Total other income, net	1,499	539	235
Net loss	(88,853)	(60,200)	(59,513)
Dividends on redeemable convertible preferred stock	—	(10,111)	—
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	—	(6,144)	—
Deemed dividend on conversion of redeemable convertible preferred stock	—	(3,837)	—
Net loss attributable to common stockholders	$(88,853)	$(80,292)	$(59,513)
Net loss per share attributable to common stockholders – basic and diluted	$(1.60)	$(3.66)	$(2.87)
Weighted average common shares outstanding – basic and diluted	55,616,092	21,917,415	20,719,761

The accompanying notes are an integral part of these consolidated financial statements.

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DICERNA PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CHANGES IN REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY
(in thousands, except share data)

	REDEEMABLE CONVERTIBLE PREFERRED STOCK		COMMON STOCK		ADDITIONAL PAID-IN CAPITAL	ACCUMULATED DEFICIT	TOTAL STOCKHOLDERS' EQUITY
	SHARES	AMOUNT	SHARES	AMOUNT			
BALANCE – January 1, 2016	—	$ —	20,594,575	$ 2	$ 287,263	$ (196,243)	$ 91,022
Exercises of common stock options and sales of common stock under Employee Stock Purchase Plan	—	—	152,200	—	561	—	561
Vesting of restricted common stock	—	—	10,000	—	—	—	—
Settlement of restricted stock for tax withholding	—	—	(3,774)	—	(27)	—	(27)
Stock-based compensation expense	—	—	—	—	9,165	—	9,165
Net loss	—	—	—	—	—	(59,513)	(59,513)
BALANCE – December 31, 2016	—	—	20,753,001	2	296,962	(255,756)	41,208
Issuance of redeemable convertible preferred stock, net of issuance costs of $750	700,000	69,250	—	—	—	—	—
Issuance of common stock from public offering, net of underwriters' commissions and offering costs of $3,221	—	—	6,571,428	1	42,778	—	42,779
Beneficial conversion feature, redeemable convertible preferred stock	—	(6,144)	—	—	6,144	—	6,144
Deemed dividend, beneficial conversion feature, and redeemable convertible preferred stock	—	6,144	—	—	(6,144)	—	(6,144)
Accretion of share issuance costs on redeemable convertible preferred stock	—	750	—	—	(750)	—	(750)
Dividends declared, redeemable convertible preferred stock	55,124	9,361	—	—	(9,361)	—	(9,361)
Conversion of redeemable convertible preferred stock	(755,124)	(79,361)	24,206,663	2	79,359	—	79,361
Exercises of common stock options and sales of common stock under Employee Stock Purchase Plan	—	—	107,523	—	290	—	290
Vesting of restricted common stock	—	—	10,000	—	—	—	—

Settlement of restricted stock for tax withholding	—	—	(3,774)	—	(11)	—	(11)
Stock-based compensation expense	—	—	—	—	7,770	—	7,770
Net loss	—	—	—	—	—	(60,200)	(60,200)
BALANCE – December 31, 2017	—	—	51,644,841	5	417,037	(315,956)	101,086
Proceeds from issuance of common stock from public offering, net of underwriters' commissions and offering costs of $330	—	—	8,832,565	1	107,769	—	107,770
Issuance of common stock to Alnylam Pharmaceuticals, Inc.	—	—	983,208	—	10,315	—	10,315
Issuance of common stock to collaboration partners	—	—	6,250,019	1	60,411	—	60,412
Exercise of warrants to purchase common stock	—	—	45,710	—	49	—	49
Exercises of common stock options and sales of common stock under Employee Stock Purchase Plan	—	—	448,173	—	2,061	—	2,061
Vesting of restricted common stock	—	—	10,000	—	—	—	—
Settlement of restricted stock for tax withholding	—	—	(3,774)	—	(35)	—	(35)
Stock-based compensation expense	—	—	—	—	7,888	—	7,888
Net loss	—	—	—	—	—	(88,853)	(88,853)
BALANCE – December 31, 2018	—	$ —	68,210,742	$ 7	$ 605,495	$ (404,809)	$ 200,693

The accompanying notes are an integral part of these consolidated financial statements.

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DICERNA PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net loss	$(88,853)	$(60,200)	$(59,513)
Adjustments to reconcile net loss to net cash used in operating activities:			
Non-cash litigation expense	10,315	—	—
Stock-based compensation expense	7,888	7,770	9,165
Depreciation and amortization expense	774	778	840
Loss on disposal of property and equipment	12	51	—
Amortization of (premium) discount on investments	(1,126)	(169)	73
Changes in operating assets and liabilities:			
Litigation settlement payable	10,500	—	—
Deferred revenue	173,916	9,270	—
Prepaid expenses and other assets	532	(1,459)	(414)
Accounts payable	(1,217)	626	1,644
Contract receivables	(100,000)	—	—
Withholding tax receivable	1,583	(1,583)	—
Accrued expenses and other liabilities	3,974	(411)	(542)
Net cash provided by (used in) operating activities	18,298	(45,327)	(48,747)
CASH FLOWS FROM INVESTING ACTIVITIES:			
Maturities of held-to-maturity investments	81,000	70,000	48,500
Purchases of held-to-maturity investments	(283,372)	(89,719)	(35,031)
Purchases of property and equipment	(359)	(133)	(449)
Net cash (used in) provided by investing activities	(202,731)	(19,852)	13,020
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from issuance of common stock, net of underwriters' commissions	108,099	43,225	—
Payments of common stock offering costs	(703)	(23)	—
Proceeds from issuance of redeemable convertible preferred stock	—	70,000	—
Redeemable convertible preferred stock issuance costs	—	(750)	—
Proceeds from issuance of common stock to collaboration partners	60,412	—	—
Proceeds from exercises of common stock warrants, stock options and issuances under Employee Stock Purchase Plan	2,110	290	561
Settlement of restricted stock for tax withholding	(35)	(11)	(27)
Net cash provided by financing activities	169,883	112,731	534
NET (DECREASE) INCREASE IN CASH, CASH EQUIVALENTS, AND RESTRICTED CASH EQUIVALENTS	(14,550)	47,552	(35,193)
CASH, CASH EQUIVALENTS, AND RESTRICTED CASH EQUIVALENTS – Beginning of year	69,533	21,981	57,174
CASH, CASH EQUIVALENTS AND RESTRICTED CASH EQUIVALENTS – End of year	$54,983	$69,533	$21,981
NONCASH INVESTING ACTIVITIES:			
Property and equipment purchases included in accounts payable and accrued expenses	$1,648	$15	$53
NONCASH FINANCING ACTIVITIES:			

Conversion of redeemable convertible preferred stock into common stock	$—	$79,361	$—
Dividends on redeemable convertible preferred stock	$—	$10,111	$—
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	$—	$6,144	$—
Deemed dividend on conversion of redeemable convertible preferred stock	$—	$3,837	$—
Common stock offering costs included in accounts payable or accrued expenses	$50	$423	$—

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The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheets that sum to the total of the same such amounts shown in the consolidated statements of cash flows:

	DECEMBER 31,		
	2018	2017	2016
Cash and cash equivalents	$54,239	$68,789	$20,865
Restricted cash equivalents	744	744	1,116
Total cash, cash equivalents, and restricted cash equivalents shown in the consolidated statements of cash flows	$54,983	$69,533	$21,981

The accompanying notes are an integral part of these consolidated financial statements.

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DICERNA PHARMACEUTICALS, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(tabular amounts in thousands, except share and per share data and where otherwise noted)

1. DESCRIPTION OF BUSINESS AND BASIS OF PRESENTATION

Business

Dicerna™ Pharmaceuticals, Inc. ("Dicerna" or the "Company"), a Delaware corporation founded in 2006 and headquartered in Cambridge, Massachusetts, is a biopharmaceutical company focused on the discovery and development of innovative subcutaneously delivered ribonucleic acid ("RNA") interference ("RNAi")-based pharmaceuticals using its GalXC™ RNAi platform for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases. Within these therapeutic areas, the Company believes its GalXC RNAi platform will allow the Company to build a broad pipeline of therapeutics with attractive pharmaceutical properties, including a subcutaneous route of administration, infrequent dosing (e.g., dosing that is monthly or quarterly, and potentially even less frequent), high therapeutic index, and specificity to a single target gene.

Basis of presentation

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") and include the accounts of Dicerna Pharmaceuticals, Inc. and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Reclassifications

Effective January 1, 2018, the Company changed the presentation of the income from government grants from the caption "Grant revenue" to an offset to research and development expenses. Also, effective April 1, 2018, the Company changed the presentation of certain litigation-related expenses associated with the litigation with Alnylam Pharmaceuticals, Inc. ("Alnylam") from the caption "General and administrative" expense to "Litigation expense." The changes associated with changes in presentation were applied retrospectively through the recast of affected prior period amounts in the consolidated statements of operations. The primary effects of such changes were:

- the reclassification of grant income from revenue to the presentation as an offset to research and development expenses of $1.1 million and $0.3 million for the years ended December 31, 2017 and 2016, respectively; and
- the reclassification of certain litigation-related expenses historically included in general and administrative expense to litigation expense in the consolidated statements of operations of $9.0 million and $2.9 million for the years ended December 31, 2017 and 2016, respectively.

Significant judgments and estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the revenues and expenses incurred during the reporting periods. On an ongoing basis, the Company evaluates its judgments and estimates, including those related to revenue recognition and accrued expenses. The Company bases its estimates on historical experience and on various other factors that the Company believes are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results could differ materially from those estimates.

Liquidity

The Company had cash, cash equivalents, and held-to-maturity investments of $302.6 million as of December 31, 2018. The Company believes that its current cash, cash equivalents, and held-to-maturity investments as of December 31, 2018 will be sufficient to fund the execution of its current clinical and operating plan beyond 2020. This estimate assumes no new funding from additional collaboration agreements or from external financing events and no significant unanticipated changes in costs and expenses. In early 2019, the Company received $5.0 million from BI for the Option Payment and $100.0 million for the upfront cash payment from the Company's recent collaboration with Eli Lilly and Company ("Lilly"), and also paid $10.5 million to Alnylam for the Confidential Settlement Agreement and General Release ("Settlement Agreement").

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2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Cash and cash equivalents

Cash and cash equivalents includes all highly liquid investments, including money market funds, maturing within 90 days from the date of purchase.

Restricted cash equivalents

Restricted cash equivalents includes the balance of funds held in a money market collateral account that is restricted to secure a letter of credit for the Company's operating lease for office and laboratory space at 87 Cambridgepark Drive in Cambridge, Massachusetts. The letter of credit is required to be maintained throughout the term of the Company's lease, which expires on November 30, 2020.

Concentrations of credit risk and significant customers

Financial instruments that subject the Company to significant concentrations of credit risk consist of cash, cash equivalents, restricted cash equivalents, held-to-maturity investments, contract receivables, and the withholding tax receivable (see Note 8 – Collaborative Research and License Agreements). All of the Company's cash, cash equivalents, restricted cash equivalents, and held-to-maturity investments are invested in money market funds or United States ("U.S.") treasury securities that management believes to be of high credit quality.

The Company's revenues for the year ended December 31, 2018 are a result of the Company's collaboration agreements with Boehringer Ingelheim ("BI") and Alexion Pharmaceuticals, Inc. ("Alexion"). BI represented substantially all of the Company's revenue from collaborative arrangements for the years ended December 31, 2018 and 2017. All revenues recognized by the Company to date were earned in the U.S.

At December 31, 2018, the balance of the Company's contract receivables was solely related to the non-refundable, non-creditable upfront payment due to the Company in connection with a collaboration agreement entered into with Lilly (see Note 8 – Collaborative Research and License Agreements). The Company did not have any contract receivables at December 31, 2017.

The Company does not currently own or operate any manufacturing facilities for the production of preclinical, clinical, or commercial quantities of any of its product candidates. For each product candidate, the Company currently contracts with manufacturers, and expects to continue to do so to meet the preclinical and clinical requirements of its product candidates. For the year ended December 31, 2018, the Company had one contract manufacturing relationship which accounted for approximately 10.9% of the Company's total purchases.

Property and equipment

Property and equipment are stated at cost. Major betterments are capitalized whereas expenditures for maintenance and repairs which do not improve or extend the life of the respective assets are charged to operations as incurred. Depreciation is provided using the straight-line method over the estimated useful lives, as shown below:

ASSET CATEGORY	ESTIMATED USEFUL LIVES
Laboratory equipment	5 years
Office and computer equipment	3 - 5 years
Furniture and fixtures	5 years
Leasehold improvements	5 years or the remaining term of lease, if shorter

Construction in process is stated at cost, which includes the cost of construction and other direct costs attributable to the construction. No provision for depreciation and amortization expense is recorded related to construction in process until the relevant assets are completed and put into use. At December 31, 2018, the balance of construction in process includes costs associated with laboratory equipment under installation and the construction of certain leasehold improvements.

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Impairment of long-lived assets

Long-lived assets are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable. When such events occur, the Company compares the carrying amounts of the assets to their undiscounted expected future cash flows. If this comparison indicates that there is an impairment, the amount of the impairment is calculated as the difference between the carrying value and fair value of the related asset. During the years ended December 31, 2018, 2017, and 2016, no impairments were recorded.

Segment and geographic information

Operating segments are defined as components (business activity from which it earns revenue and incurs expenses) of an enterprise about which discrete financial information is available and regularly reviewed by the chief operating decision maker in deciding how to allocate resources and in assessing performance. The Company, through its Chief Executive Officer in his role as chief operating decision maker, views its operations and manages its business as one operating segment. All long-lived assets of the Company are located in the United States.

Research and development costs

Research and development costs consist of expenses incurred in performing research and development activities, including compensation and benefits for full-time research and development employees, an allocation of facility expenses, overhead expenses and other outside expenses. Research and development costs are expensed as incurred. Research and development costs that are paid in advance of performance are deferred as a prepaid expense and amortized over the service period as the services are provided. The Company records grants from governmental and non-profit agencies as a reduction in research and development expense. Grants are recognized when there is reasonable assurance that the Company will comply with the conditions attached to the grant arrangement and the grant will be received. Grant payments received related to research and development costs incurred prior to the approval of the qualifying program are recognized immediately upon approval of the program by the grantor.

Revenue recognition

The Company generates revenue from research collaboration and license agreements with customers. Goods and services in the agreements may include the grant of licenses for the use of the Company’s technology, the provision of services associated with the research and development of product candidates, manufacturing services, and participation on joint steering committees. Such agreements may provide for consideration to the Company in the form of upfront payments; funding or reimbursement of research and development services; reimbursement of certain costs; option exercise payments; payments due upon the achievement of preclinical, clinical, regulatory, and sales-based milestones; and royalty payments on licensed products.

On January 1, 2018, the Company adopted the new revenue recognition standard, discussed below under the heading “Recent accounting pronouncements,” which amended revenue recognition principles and provides a single, comprehensive set of criteria for revenue recognition. The new revenue standard applies to all contracts with customers except for contracts that are within the scope of other standards. The new guidance provides a five-step framework through which revenue is recognized when control of promised goods or services is transferred to a customer at an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To determine revenue recognition for arrangements that the Company concludes are within the scope of the new revenue recognition standard, management performs the following five steps: (i) identifies the contract(s) with a customer; (ii) identifies the performance obligations in the contract; (iii) determines the transaction price, including whether there are any constraints on variable consideration; (iv) allocates the transaction price to the performance obligations; and (v) recognizes revenue when (or as) the Company satisfies a performance obligation. At contract inception, once a contract is determined to be within the scope of the new revenue standard, Dicerna assesses whether individual goods or services promised within each contract are distinct and, therefore, represent a separate performance obligation. Goods and services that are determined not to be distinct are combined with other promised goods and services until a distinct bundle is identified. Dicerna allocates the transaction price (the amount of consideration to which the Company expects to be entitled in exchange for the promised goods or services) to each performance obligation and recognizes the associated revenue when (or as) each performance obligation is satisfied. The Company’s estimate of the transaction price for each contract includes all variable consideration to which Dicerna expects to be entitled at each measuring period.

When two or more contracts are entered into with the same customer at or near the same time, the Company evaluates the contracts to determine whether the contracts should be accounted for as a single arrangement. Contracts are combined and accounted for as a single arrangement if one or more of the following criteria are met: (i) the contracts are negotiated as a package with a single commercial objective; (ii) the amount of consideration to be paid in one contract depends on the price or performance of the other contract; or (iii) the goods or services promised in the contracts (or some goods or services promised in each of the contracts) are a single performance obligation.

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The evaluation of whether promised goods or services represent distinct performance obligations is subjective and requires the Company to make judgments about the promised goods and services and whether such goods and services are separable from the other aspects of the contract(s).

The transaction price is allocated among the performance obligations on a relative standalone selling price basis, and the applicable revenue recognition criteria are applied to each of the separate performance obligations. The Company may estimate the standalone selling price using a residual method when the selling price is highly variable because a representative standalone selling price is not discernible from past transactions or other observable evidence, or when the selling price is uncertain.

Determining the standalone selling price for performance obligations requires significant judgment. When an observable price of a promised good or service is not readily available, the Company considers relevant assumptions to estimate the standalone selling price, including, as applicable, market conditions, development timelines, probabilities of technical and regulatory success, reimbursement rates for personnel costs, forecasted revenues, potential limitations to the selling price of the product, and discount rates.

The Company applies judgment in determining whether a combined performance obligation is satisfied at a point in time or over time, and, if over time, concluding upon the appropriate method of measuring progress to be applied for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, as estimates related to the measure of progress change, related revenue recognition is adjusted accordingly. Changes in the Company's estimated measure of progress are accounted for prospectively as a change in accounting estimate.

The Company receives payments from its licensees as established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt or when due and most often require deferral of revenue recognition to a future period until the Company performs its obligations under the underlying arrangements. Where applicable, amounts are recorded as contracts receivable when the Company's right to consideration is unconditional.

Licenses of intellectual property: If a license granted to a customer to use the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue from consideration allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company applies judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, to conclude upon the appropriate method of measuring progress for purposes of recognizing revenue related to consideration allocated to the performance obligation.

Research and development services: Arrangements that include a promise for the Company to provide research or development services are assessed to determine whether the services are capable of being distinct, are not highly interdependent or do not significantly modify one another, and if so, the services are accounted for as a separate performance obligation as the services are provided to the customer. Otherwise, when research or development services are determined not to be capable of being distinct, such services are added to the performance obligation that includes the underlying license. For research and development services that are bundled with other promises, the Company applies judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, to conclude upon the appropriate method of measuring progress for purposes of recognizing revenue related to consideration allocated to the performance obligation. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

Options: Customer options, such as options granted to allow a licensee to choose to research and develop additional or reserve product candidates against target genes to be identified in the future, or options that allow a customer to designate a target as a lead product, are evaluated at contract inception in order to determine whether those options provide a material right (i.e., an optional good or service offered for free or at a discount) to the customer. If the customer options represent a material right, the material right is treated as a separate performance obligation at the outset of the arrangement. The Company allocates the transaction price to material rights based on the standalone selling price, and revenue is recognized when or as the future goods or services are transferred or when the option expires. Customer options that are not material rights do not give rise to a separate performance obligation, and as

such, the additional consideration that would result from a customer exercising an option in the future is not included in the transaction price for the current contract. Instead, the option is deemed a marketing offer, and additional option fee payments are recognized or begin being recognized as revenue when the licensee exercises the option. The exercise of an option that does not represent a material right is treated as a separate contract for accounting purposes. Milestone payments: At the inception of each contract with a customer that includes development or regulatory milestone payments, the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If the Company concludes it is probable that a significant revenue reversal

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would not occur, the associated milestone payment is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are generally not considered probable of being achieved until those approvals are received. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied.

At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of all milestones and any related constraints, and, if necessary, adjusts the estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis and are recorded as revenue and through earnings in the period of adjustment.

Royalties: For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and when the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Contract costs: The Company recognizes as an asset the incremental costs of obtaining a contract with a customer if the costs are expected to be recovered. The Company has elected a practical expedient wherein it recognizes the incremental costs of obtaining a contract as an expense when incurred if the amortization period of the asset that it otherwise would have recognized is one year or less. To date, the Company has not incurred any incremental costs of obtaining a contract with a customer.

Contract modifications: Contract modifications, defined as changes in the scope or price (or both) of a contract that are approved by the parties to the contract, such as a contract amendment, exist when the parties to a contract approve a modification that either creates new or changes existing enforceable rights and obligations of the parties to the contract. Depending on facts and circumstances, the Company accounts for a contract modification as one of the following: (i) a separate contract; (ii) a termination of the existing contract and a creation of a new contract; or (iii) a combination of the preceding treatments. A contract modification is accounted for as a separate contract if the scope of the contract increases because of the addition of promised goods or services that are distinct and the price of the contract increases by an amount of consideration that reflects the Company’s standalone selling prices of the additional promised goods or services. When a contract modification is not considered a separate contract and the remaining goods or services are distinct from the goods or services transferred on or before the date of the contract modification, the Company accounts for the contract modification as a termination of the existing contract and a creation of a new contract. When a contract modification is not considered a separate contract and the remaining goods or services are not distinct, the Company accounts for the contract modification as an add-on to the existing contract and as an adjustment to revenue on a cumulative catch-up basis.

The Company receives payments from its licensees as established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt or when due and may require deferral of revenue recognition to a future period until the Company performs its obligations under these arrangements. Where applicable, amounts are recorded as contracts receivable when the Company’s right to consideration is unconditional. The Company does not assess whether a contract with a customer has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less.

Stock-based compensation

The Company’s stock-based compensation cost is measured at the grant date of the stock-based award based on the fair value of the award and is recognized as expense over the requisite service period, which generally represents the vesting period, and includes an estimate of the awards that will be forfeited. The Company uses the Black-Scholes valuation model for estimating the fair value of stock options. The fair value of stock option awards is affected by the valuation assumptions, including the expected volatility based on comparable market participants, expected term of the option, risk-free interest rate and expected dividends.

Income taxes

The Company records deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the Company’s financial statement carrying amounts and the tax basis of assets and liabilities

using enacted tax rates expected to be in effect in the years in which the differences are expected to reverse. A valuation allowance is provided to reduce the net deferred tax assets to the amount that will more likely than not be realized.

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The Company also assesses the probability that the positions taken or expected to be taken in its income tax returns will be sustained by taxing authorities. A “more likely than not” (more than 50 percent) recognition threshold must be met before a tax benefit can be recognized. Tax positions that are more likely than not to be sustained are reflected in the Company’s consolidated financial statements. Tax positions are measured as the largest amount of tax benefit that is greater than 50 percent likely of being realized upon settlement with a taxing authority that has full knowledge of all relevant information. The difference between the benefit recognized for a position and the tax benefit claimed on a tax return is referred to as an unrecognized tax benefit. Potential interest and penalties associated with such uncertain tax positions are recorded as a component of income tax expense.

Net loss per common share attributable to common stockholders

The Company computes basic net loss per common share by dividing net loss attributable to common stockholders by the weighted average number of common shares outstanding. In periods of net income, the Company’s accounting policy includes allocating a proportional share of net income to participating securities, as determined by dividing total weighted average participating securities by the sum of the total weighted average common shares and participating securities (the “two-class method”). The Company’s nonvested restricted shares participated in any dividends declared by the Company and were therefore considered to be participating securities. Participating securities have the effect of diluting both basic and diluted earnings per share during periods of income. During periods when the Company incurred a net loss, the Company did not allocate a loss to participating securities because they had no contractual obligation to share in the losses of the Company. The Company computes diluted net loss per common share after giving consideration to the dilutive effect of stock options, warrants, nonvested restricted stock, and redeemable convertible preferred shares that are outstanding during the period, except where such non-participating securities would be anti-dilutive.

The outstanding securities presented below were excluded from the calculation of net loss per share attributable to common stockholders because such securities would have been anti-dilutive due to the Company’s net loss per share attributable to common stockholders during the periods ending on the dates presented.

	DECEMBER 31,		
	2018	2017	2016
Options to purchase common stock	7,787,690	6,124,096	5,099,449
Warrants to purchase common stock	2,198	87,901	87,901
Nonvested restricted common stock	—	10,000	20,000
Total	7,789,888	6,221,997	5,207,350

Comprehensive loss

Comprehensive loss is defined as the change in stockholders’ equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. The Company has no comprehensive loss items other than net loss.

Recent accounting pronouncements

The following table provides a description of the recent accounting pronouncements that may have a material effect on the Company’s consolidated financial statements:

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Standard	Description	Effective Date for Company	Effect on the Consolidated Financial Statements
Accounting Standards Adopted During the Year Ended December 31, 2018			
ASU 2014-09, Revenue from Contracts with Customers (Topic 606) and related amendments ("ASC 606")	This ASU amends the guidance for accounting for revenue from contracts with customers, superseding the revenue recognition requirements in ASC 605, Revenue Recognition. ASC 606 was effective for annual reporting periods beginning after December 15, 2017. Under ASC 606, two adoption methods were allowed: retrospectively to all prior reporting periods presented, with certain practical expedients permitted, or retrospectively with the cumulative effect of initially adopting ASC 606 recognized at the date of initial application.	January 1, 2018	Effective January 1, 2018, the Company adopted the requirements of ASC 606 using the full retrospective method, which required the Company to recast the prior reporting periods presented. All financial statements and disclosures have been recast to comply with ASC 606. See "Change in accounting principle" below for a summary of the amounts by which each financial statement line item was affected by the adoption of ASC 606. The adoption of ASC 606 has also resulted in additional revenue-related disclosures in the notes to the Company's consolidated financial statements (see Note 8 – Collaborative Research and License Agreements).
ASU No. 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash ("ASU 2016-18")	In November 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash ("ASU 2016-18"), a consensus of the FASB's EITF. ASU 2016-18 requires that the statement of cash flows explain the change during the period in the total of cash and cash equivalents, including amounts generally described as restricted cash or restricted cash equivalents. Entities are required to reconcile such total to amounts on the balance sheet and disclose the nature of the restrictions. By requiring that the statement of cash flows explain the change during the period in the total of cash, cash equivalents and restricted cash, the new guidance eliminates current diversity in practice.	January 1, 2018	The Company adopted ASU 2016-18 on January 1, 2018 and applied this new guidance retrospectively to all periods presented. Consequently, transfers between restricted and unrestricted cash equivalents accounts are no longer reported as a cash flow in the Company's consolidated statement of cash flows. As a result of the adoption of this standard, the Company includes its restricted cash equivalents balance in the cash and cash equivalents reconciliation of operating, investing and financing activities. The retrospective adoption resulted in the inclusion of restricted cash equivalents of $0.7 million, and $1.1 million in the consolidated statements of cash flows as of December 31, 2017 and 2016, respectively.

Standard	Description	Effective Date for Company	Effect on the Consolidated Financial Statements
Recently Issued Accounting Standards Not Yet Adopted			
ASU 2016-02, Leases (Topic	This ASU supersedes existing accounting standards for lease accounting, including requiring lessees to recognize most leases on their balance sheets and making targeted changes to lessor	January 1, 2019	Management expects that the adoption of ASU 2016-02 will result in the recognition of a right of use asset and related liability associated with the Company's

842) accounting. ASU 2016-02 will be effective for the Company beginning in the first quarter of 2019. ASU 2016-02 requires a modified retrospective transition approach for all leases existing at, or entered into after, the date of initial application, with an option to use certain transition relief. In July 2018, the FASB issued ASU 2018-11, Leases (Topic 842): Targeted Improvements ("ASU 2018-11"), which allows entities to initially apply the new lease guidance at the adoption date and recognize a cumulative-effect adjustment to the opening balance of retained earnings in the period of adoption.

non-cancelable operating lease arrangements for office and laboratory spaces (see Note 14 – Commitments and Contingencies and Note 17 - Subsequent Events). The Company is in the process of determining whether it will utilize the optional transition method presented in ASU 2018-11.

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Change in accounting principle

In May 2014, the Financial Accounting Standards Board ("FASB") issued ASC 606. Under the standard, revenue is recognized when a customer obtains control of promised goods or services in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. Effective January 1, 2018, the Company adopted ASC 606 using the full retrospective method, which required the Company to recast the prior reporting periods presented.

The Company has recast its consolidated financial statements from amounts previously reported due to the adoption of ASC 606. Select Consolidated Statement of Operations line items, which reflect the impact of the adoption of ASC 606, are as follows:

	YEAR ENDED DECEMBER 31, 2017		
	AS REPORTED	ADJUSTMENTS	AS ADJUSTED
Revenue from collaborative arrangements	$1,182	$ (152)	$ 1,030
Loss from operations	$(60,587)	$ (152)	$ (60,739)
Net loss	$(60,048)	$ (152)	$ (60,200)
Net loss attributable to common stockholders	$(80,140)	$ (152)	$ (80,292)

The adoption of ASC 606 did not have an impact on net loss per share attributable to common stockholders for any period presented.

Select Consolidated Balance Sheet line items, which reflect the adoption of ASC 606, are as follows:

	DECEMBER 31, 2017		
	AS REPORTED	ADJUSTMENTS	AS ADJUSTED
Prepaid expenses and other current assets	$3,297	$ 118	$ 3,415
Current portion of deferred revenue	$6,000	$ 180	$ 6,180
Deferred revenue, net of current portion	$3,000	$ 90	$ 3,090
Accumulated deficit	$(315,804)	$ (152)	$ (315,956)

The adoption of ASC 606 did not have an impact on net cash used in operating, investing, or financing activities in the Company’s Consolidated Statements of Cash Flows.

As the Company did not have any revenue from collaborative arrangements during the year ended December 31, 2016, the adoption of ASC 606 did not have an impact on the beginning balance of accumulated deficit for the earliest period presented in the Consolidated Statements of Changes in Redeemable Convertible Preferred Stock and Stockholders’ Equity.

3. HELD-TO-MATURITY INVESTMENTS

The Company invests its excess cash balances in short-term and long-term fixed-income investments. The Company determines the appropriate classification of investments at the time of purchase and re-evaluates such designation as of each balance sheet date. Debt securities carried at amortized cost are classified as held-to-maturity when the Company has the positive intent and ability to hold the securities to maturity.

The Company’s investment policy mandates that, at the time of purchase, the maturity of each investment within its portfolio shall not exceed 24 months. In addition, the weighted average maturity of the investment portfolio must not exceed 12 months.

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The following tables provide information relating to the Company's held-to-maturity investments:

	DECEMBER 31, 2018			
DESCRIPTION	AMORTIZED COST	GROSS UNREALIZED HOLDING GAINS	GROSS UNREALIZED HOLDING LOSSES	FAIR VALUE
U.S. Treasury securities maturing in one year or less	$248,387	$ —	$ (43)	$248,344

	DECEMBER 31, 2017			
DESCRIPTION	AMORTIZED COST	GROSS UNREALIZED HOLDING GAINS	GROSS UNREALIZED HOLDING LOSSES	FAIR VALUE
U.S. Treasury securities maturing in one year or less	$44,889	$ —	$ (30)	$44,859

4. FAIR VALUE MEASUREMENTS

Fair value is an exit price, representing the amount that would be received from the sale of an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. Valuation techniques used to measure fair value are performed in a manner to maximize the use of observable inputs and minimize the use of unobservable inputs. As a basis for considering such assumption the accounting literature establishes a three-tier value hierarchy which prioritizes the inputs used in measuring fair value as follows:

- Level 1 – observable inputs, such as quoted prices in active markets;
- Level 2 – inputs other than the quoted prices in active markets that are observable either directly or indirectly; and
- Level 3 – unobservable inputs for which there is little or no market data, which requires the Company to develop its own assumptions.

A summary of the Company's assets that are measured or disclosed at fair value on a recurring basis is presented below:

	DECEMBER 31, 2018			
DESCRIPTION	TOTAL FAIR VALUE	LEVEL 1	LEVEL 2	LEVEL 3
Cash equivalents				
Money market funds	$44,886	$44,886	$—	$ —
Held-to-maturity investment				
U.S. Treasury securities	248,344	—	248,344	—
Restricted cash equivalents				
Money market funds	744	—	744	—
Total	$293,974	$44,886	$249,088	$ —

	DECEMBER 31, 2017			
DESCRIPTION	TOTAL FAIR VALUE	LEVEL 1	LEVEL 2	LEVEL 3
Cash equivalents				
Money market funds	$51,441	$51,441	$—	$ —
Held-to-maturity investments				
U.S. Treasury securities	44,859	—	44,859	—
Restricted cash equivalents				
Money market funds	744	—	744	—
Total	$97,044	$51,441	$45,603	$ —

The Company’s cash equivalents, which are in money market funds, are classified within Level 1 of the fair value hierarchy because they are valued using quoted prices in active markets as of December 31, 2018 and 2017.

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The Company's held-to-maturity investments and restricted cash equivalents bore interest at the prevailing market rates for instruments with similar characteristics and therefore approximated fair value. These financial instruments were classified within Level 2 of the fair value hierarchy, because the inputs to the fair value measurement are valued using observable inputs as of December 31, 2018 and 2017.

As of December 31, 2018 and 2017, the Company's contract receivables, accounts payable, and accrued expenses approximated their estimated fair values because of the short-term nature of these financial instruments. As of December 31, 2017, the carrying amount of the withholding tax receivable also approximated its estimated fair value due to the short-term nature of the instrument.

As of December 31, 2018, the Company had a remaining cash obligation of $10.5 million payable to Alnylam (see Note 15). Upon receipt of certain upfront cash payment owed to the Company resulting from signing the Lilly Collaboration Agreement in October 2018, the Company anticipates that the cash obligation will be payable in the first quarter of 2019 and has therefore adjusted the liability equal to the estimated present value of the obligation of $10.5 million and included the obligation in current liabilities at December 31, 2018. As the present value of the litigation settlement payable at December 31, 2018 was determined using market rates based on the nature of the obligation and the Company's creditworthiness, the carrying value approximates the fair value. There was no liability recorded related to the settlement as of December 31, 2017.

The Company's policy is to recognize transfers between levels of the fair value hierarchy, if any, at the end of the reporting period; however, there have been no such transfers during any of the periods presented.

5. PREPAID EXPENSES AND OTHER CURRENT ASSETS

Prepaid expenses and other current assets consist of the following:

	DECEMBER 31,	
	2018	2017
Prepaid clinical, contract research, and manufacturing costs	$ 1,419	$ 1,931
Interest receivable and other current assets	815	391
Prepaid insurance	341	318
Prepaid rent	245	239
Other	68	536
Prepaid expenses and other current assets	$ 2,888	$ 3,415

6. PROPERTY AND EQUIPMENT, NET

Property and equipment, net, consists of the following:

	DECEMBER 31,	
	2018	2017
Laboratory equipment	$4,607	$4,410
Office and computer equipment	1,021	900
Furniture and fixtures	479	479
Leasehold improvements	257	257
Construction in process	1,661	—
Property and equipment, at cost	8,025	6,046
Less accumulated depreciation and amortization	(5,307)	(4,534)
Property and equipment, net	$2,718	$1,512

Depreciation and amortization expense was $0.8 million for each of the years ended December 31, 2018, 2017, and 2016.

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7. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other current liabilities consist of the following:

	DECEMBER 31,	
	2018	2017
Accrued clinical, contract research, and manufacturing costs	$ 3,960	$ 1,860
Accrued compensation and related benefits	3,684	1,987
Accrued professional fees	1,693	1,488
Accrued other expenses	312	391
Accrued expenses and other current liabilities	$ 9,649	$ 5,726

8. COLLABORATIVE RESEARCH AND LICENSE AGREEMENTS

Lilly collaboration and share purchase agreements

On October 25, 2018, the Company entered into a Collaboration and License Agreement (the "Lilly Collaboration Agreement") with Lilly for the discovery, development, and commercialization of potential new medicines in the areas of cardiometabolic disease, neurodegeneration, and pain. Under the terms of the Lilly Collaboration Agreement, the Company and Lilly will seek to use the Company's proprietary GalXC RNAi technology platform to progress new drug targets toward clinical development and commercialization. In addition, the Company and Lilly will collaborate to extend the GalXC RNAi platform technology to non-liver (i.e., non-hepatocyte) tissues, including neural tissues. The Lilly Collaboration Agreement provides that the Company will work exclusively with Lilly in the neurodegeneration and pain fields, with the exception of mutually agreed upon orphan indications. Additionally, the Company will work exclusively with Lilly on select targets in the cardiometabolic field. Under the Lilly Collaboration Agreement, the Company will provide Lilly with exclusive and non-exclusive licenses to support the companies' activities and to enable Lilly to commercialize products derived from or containing compounds developed pursuant to such agreement. The Lilly Collaboration Agreement provides for three initially named hepatocyte targets, and the Company and Lilly have agreed to develop an initial research program with the goal of researching and developing multiple lead candidates directed to each of these initial targets. The Lilly Collaboration Agreement contemplates in excess of 10 targets.

Under the terms of the Lilly Collaboration Agreement, Lilly agreed to pay the Company a non-refundable, non-creditable upfront payment of $100.0 million. The Company is also eligible to receive up to $350.0 million, per target, in development and commercialization milestones, in addition to a $5.0 million payment, which will become due for each of the non-hepatocyte targets when a product candidate achieves proof of principle in an animal model. In addition, the Company is eligible to earn mid-single to low-double digit royalties on product sales on a country-by-country and product-by-product basis until the later of expiration of patent rights in a country, the expiration of data or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement. Simultaneously with the entry into the Lilly Collaboration Agreement, the Company and Lilly entered into a Share Purchase Agreement (the "Lilly Share Issuance Agreement"), pursuant to which Lilly purchased 5,414,185 shares of the Company's common stock at $18.47 per share, for an aggregate purchase price of $100.0 million. Management concluded that the Lilly Share Issuance Agreement is to be combined with the Lilly Collaboration Agreement (together, the "Combined Agreements") for accounting purposes. Of the total $200.0 million upfront compensation, the Company applied equity accounting guidance to measure the $51.3 million recorded in equity upon the issuance of the shares, and $148.7 million was identified as the transaction price allocated to the revenue arrangement. The Combined Agreements were subject to customary closing conditions and clearances, including clearance under the Hart-Scott-Rodino Antitrust Improvements Act, and closed in December 2018.

The Company concluded that Lilly is a customer in this arrangement, and as such, the element of the arrangement unrelated to the issuance of the shares falls within the scope of the revenue recognition guidance. The Company identified contract promises under the Combined Agreements for licenses of intellectual property and know-how rights, associated research and development services for targets and for a new platform, and participation on a joint steering committee. The Company determined that the performance obligations were not separately identifiable and were not distinct or distinct within the context of the contract due to the specialized nature of the services to be

provided by Dicerna, specifically with respect to the Company's therapeutic expertise related to RNAi and the Company's GalXC conjugates, and the interdependent relationship between the performance obligations. As such, the Company concluded that there is a single identified combined performance obligation.

The Company used the most likely amount method to estimate variable consideration and estimated that the most likely amount for each potential development milestone payment under this agreement, which is considered variable consideration, was zero, as the achievement of those milestones is uncertain and highly susceptible to factors outside of the Company's control. Accordingly, all such

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milestones were excluded from the transaction price. Management will re-evaluate the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur and adjust the transaction price as necessary. Sales-based royalties, including milestone payments based on the level of sales, were also excluded from the transaction price, as the license is deemed to be the predominant item to which the royalties relate. The Company will recognize such revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). Revenue associated with the performance obligation will be recognized as services are provided using a cost-to-cost measure of progress method. The transfer of control occurs over time and, in management's judgment, this input method is the best measure of progress towards satisfying the performance obligation and reflects a faithful depiction of the transfer of goods and services.

No revenue was recognized under the Lilly Collaboration Agreement during the year ended December 31, 2018. The aggregate amount of the consideration received and the amount billed under the arrangement that were allocated to the revenue element of the arrangement as of December 31, 2018 relates to the Company's wholly unsatisfied performance obligation. This amount is recorded as a contract liability presented in deferred revenue at December 31, 2018 is $148.7 million, of which $54.0 million is included in the current portion of deferred revenue. As of December 31, 2018, the Company expected to recognize this amount over the remaining research term of the agreement, which is expected to extend through the first quarter of 2022, with the majority being recognized through the fourth quarter of 2021.

Alexion collaboration and equity agreements

On October 22, 2018, the Company and Alexion Pharma Holding Unlimited Company ("Alexion Pharma Holding"), an affiliate of Alexion Pharmaceuticals, Inc. ("Alexion Pharmaceuticals" and, together with Alexion Pharma Holding, "Alexion") entered into a Collaborative Research and License Agreement (the "Alexion Collaboration Agreement"). The Alexion Collaboration Agreement is for the joint discovery and development of RNAi therapies for complement-mediated diseases. Under the terms of the Alexion Collaboration Agreement, the Company and Alexion will collaborate on the discovery and development of subcutaneously delivered GalXC candidates, currently in preclinical development, for the treatment of complement-mediated diseases with potential global commercialization by Alexion. The Company will lead the joint discovery and research efforts through the preclinical stage, and Alexion will lead development efforts beginning with Phase 1 studies. The Company will be responsible for manufacturing of the GalXC candidates through the completion of Phase 1, and the related costs will be paid by Alexion. Alexion will be solely responsible for the manufacturing of any product candidate subsequent to the completion of Phase 1. The Alexion Collaboration Agreement provides Alexion with exclusive worldwide licenses as well as development and commercial rights for two of the Company's preclinical, subcutaneously delivered GalXC RNAi candidates and an exclusive option for the discovery and development of GalXC RNAi candidates against two additional complement pathway targets.

Under the terms of the Alexion Collaboration Agreement, Alexion agreed to pay the Company a non-refundable, non-reimbursable, and non-creditable upfront payment of $22.0 million. The Alexion Collaboration Agreement also provides for potential additional payments to the Company of up to $600.0 million from proceeds from target option exercises and development and sales milestones, as defined in the agreement, which is comprised of: (i) option exercise fees of up to $20.0 million, representing $10.0 million for each of the targets selected; (ii) development milestones of up to $105.0 million for each product; and (iii) aggregate sales milestones of up to $160.0 million. Under the agreement, Alexion also agreed to pay to the Company mid-single to low-double digit royalties on potential product sales on a country-by-country, product-by-product basis until the later of the expiration of patent rights in a country, the expiration of market or regulatory exclusivity in such country, or 10 years after the first product sale in such country, subject to certain royalty step-down provisions set forth in the agreement.

Simultaneously with the entry into the Alexion Collaboration Agreement, the Company and Alexion Pharmaceuticals entered into a Share Purchase Agreement (the "Alexion Share Issuance Agreement"), pursuant to which Alexion Pharmaceuticals purchased 835,834 shares of the Company's common stock at $17.95 per share at issuance, for an aggregate stated purchase price of $15.0 million. Management concluded that the Alexion Share Issuance Agreement is to be combined with the Alexion Collaboration Agreement (together, the "Alexion Agreements") for accounting

purposes. With respect to the $15.0 million of cash received upon issuance of the shares, the Company applied equity accounting guidance to measure the $9.1 million recorded in equity upon the issuance of the shares, and the remaining $5.9 million was included as a component of the transaction price attributable to the revenue arrangement.

Alexion selected two targets upon entry into the Alexion Collaboration Agreement, which, as noted above, provides Alexion with the option to select up to two additional targets, in exchange for an option fee payment of $10.0 million for each selected target.

The Company concluded that Alexion is a customer in this arrangement, and as such, the element of the arrangement unrelated to the issuance of the shares falls within the scope of the revenue recognition guidance. The Company identified the following promises under the arrangement: (i) the granting of licenses of intellectual property and know-how rights; (ii) the option to select additional targets; (iii) the option to perform validation testing on additional targets; (iv) associated research and development services for the

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initial and, as applicable, additional targets; and (v) the Company's participation in the joint steering committee. The Company concluded that the research and development services were not capable of being distinct from the research and development license, and were not distinct within the context of the contract, and should therefore be combined into a single performance obligation for each program. The Company considered the level of Alexion's therapeutic expertise specifically related to RNAi, as well as Alexion's know-how of the Company's GalXC conjugates, and concluded that Alexion cannot currently benefit from the granted license on its own or together with other resources that are readily available to Alexion, including relationships with oligonucleotide vendors who synthesize GalXC conjugates under contract with the Company. As a result, the combination of the license of intellectual property together with the provision of research and development services together represent the highest level of goods and services that can be deemed distinct.

Additionally, the Company determined that the options to select additional targets and to perform validation testing on additional targets were not priced at a discount and, as such, do not provide Alexion with material rights. Based on management's assessments, the Company identified a single performance obligation, namely, the combined license and research and development services, for each of the two initially nominated targets.

At the outset of the Alexion Collaboration Agreement, the transaction price was determined to be $37.4 million, which is comprised of the $22.0 million upfront payment, the $5.9 million identified upon issuance of the shares, as described above, and $9.5 million in aggregate contingent milestone payments that were either received or probable of achievement and under the Company's control.

The Company used the most likely amount method to estimate variable consideration and estimated that the most likely amount for each potential development milestone payment beyond the three initial research program milestones under this agreement was zero, as the achievement of those milestones is uncertain and highly susceptible to factors outside of the Company's control. Accordingly, such milestones were excluded from the transaction price. Management will re-evaluate the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur and adjust the transaction price as necessary. Sales-based royalties, including milestone payments based on the level of sales, were also excluded from the transaction price, as the license is deemed to be the predominant item to which the royalties relate. The Company will recognize such revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Revenue associated with the performance obligations is being recognized as services are provided using an input method based on a cost-to-cost measure of progress method. The transfer of control occurs over time and, in management's judgment, this input method is the best measure of progress towards satisfying the performance obligation and reflects a faithful depiction of the transfer of goods and services.

During the year ended December 31, 2018, the Company recognized $0.1 million as revenue under the Alexion Agreements in the accompanying consolidated statement of operations. The aggregate amount of the transaction price allocated to the Company's partially unsatisfied performance obligations and recorded as deferred revenue at December 31, 2018 is $31.3 million, of which $11.7 million is included in current portion of deferred revenue. As of December 31, 2018, the Company expects to recognize this amount over the remaining research program term, which is estimated to extend through the fourth quarter of 2023, with the majority being recognized through the fourth quarter of 2021.

BI Agreement and related amendments

On October 27, 2017, the Company entered into a collaborative research and license agreement with BI (the "BI Agreement"), pursuant to which the Company and BI jointly research and develop product candidates for the treatment of chronic liver disease using the GalXC platform, Dicerna's proprietary RNAi-based technology. The BI Agreement is for the development of product candidates against one target gene with an option for BI to add the development of product candidates that target a second gene (the "Second Target"). Pursuant to the BI Agreement, Dicerna granted BI a worldwide license in connection with the research and development of such product candidates and transferred certain intellectual property rights of the selected product candidates to BI for clinical development and commercialization. Dicerna also may provide assistance to BI in order to help BI further develop selected product candidates. Under the terms of the BI Agreement, BI agreed to pay Dicerna a non-refundable upfront payment of $10.0 million for the first

target, less a refundable withholding tax in Germany of $1.6 million. BI also agreed to reimburse Dicerna certain third-party expenses of $0.3 million. The German withholding tax was withheld by BI and remitted to the German tax authorities in accordance with local tax law. The Company received reimbursement of this tax in July 2018.
During the term of the research program, BI will reimburse Dicerna the cost of certain materials and third-party expenses that have been included in the preclinical studies. The Company is eligible to receive up to $191.0 million in potential development and commercial milestones related to the initial target. Dicerna is also eligible to receive royalty payments on potential global net sales, subject to certain adjustments, tiered from high single digits up to low double-digits. BI's Second Target option provided for an option fee payment of $5.0 million and success-based development and commercialization milestones and royalty payments to Dicerna.

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Milestone payments that are contingent upon the Company's performance under the BI Agreement include potential developmental milestones totaling $99.0 million. The Company has excluded these amounts from allocable consideration at the outset of the arrangement, as described below. All potential net sales milestones, totaling $95.0 million, will be accounted for in the same manner as royalties and recorded as revenue at the later of the achievement of the milestone or the satisfaction of the performance obligation.

The Company concluded that BI is a customer in this arrangement, and as such, the arrangement falls within the scope of the revenue recognition guidance. The Company identified the following performance obligations under the contract: the license of intellectual property and conducting agreed-upon research program services. The Company concluded that the license and research and development services are not capable of being distinct and are not distinct within the context of the contract; therefore, the Company considers these to be one performance obligation. The Company concluded the option underlying the transfer of future licenses and potential associated research for any not-yet-known target gene is not a performance obligation of the contract at inception because the option fee reflects the standalone selling price of the option, and therefore, the option is not considered to be a material right. The Company considered the level of BI's therapeutic expertise specifically related to RNAi, as well as BI's know-how with regard to the Company's GalXC conjugates, and concluded that BI cannot currently benefit from the granted license on its own or together with other resources that are readily available to BI, including relationships with oligonucleotide vendors who synthesize GalXC conjugates under contract with the Company. As a result, the combination of the license of intellectual property together with the provision of research and development support services together represent the highest level of goods and services that can be deemed distinct.

Based on management's evaluation, the $10.0 million non-refundable upfront fee and the $0.3 million agreed-upon reimbursable third-party expenses constituted the amount of the consideration to be included in the transaction price and was allocated to the performance obligation identified. None of the development milestones have been included in the transaction price during the period, since none of such milestone amounts are within the control of the Company and are not considered probable to occur until confirmed by BI, at BI's sole discretion. Any consideration related to commercial sales-based milestones (including royalties) will be recognized when the related sales occur, since these amounts have been determined to relate predominantly to the license granted to BI and therefore are recognized at the later of when the performance obligation is satisfied or when the related sales occur. The Company re-evaluates the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur and adjusts the transaction price as necessary.

The $10.3 million transaction price is recognized over the research term, currently estimated to extend through August 2019, which represents the Company's current best estimate of the period of the obligation to provide research support services to BI. Related revenue is recognized on a straight-line basis, which is in management's judgment an appropriate measure of progress toward satisfying the performance obligation, largely in absence of evidence that obligations are fulfilled in a specific pattern.

BI contract amendment

In October 2018, BI exercised its Second Target option, which entitles the Company to a non-refundable payment of $5.0 million upon the agreement of a research work plan and budget for the Second Target. The terms of the Second Target option exercise and related rights and obligations associated with the Second Target were agreed between the Company and BI in an Additional Target Agreement (the "ATA"), which was entered into on December 31, 2018. Under the terms of the ATA, BI will be responsible for future clinical development and commercialization of candidate products for the Second Target. Additionally, during the term of the research program, BI will reimburse the Company for certain expenses. The Company is eligible to receive up to $170.0 million in potential development and commercial milestones related to the Second Target. The Company is also eligible to receive tiered royalty payments on potential global net sales, subject to certain adjustments, in the mid-single digits. Except as otherwise set forth in the ATA, development of the Second Target is subject to the terms of the original BI Agreement.

Management determined that the addition of the Second Target upon exercise of the Second Target Option resulted in a new contract for accounting purposes, and the $5.0 million exercise price was representative of the standalone selling price. The exercise of the Second Target option on December 31, 2018 through the ATA created a new contract for accounting purposes. Consistent with the reasons described related to the initial target, management

concluded that the non-refundable Second Target option exercise fee (akin to an upfront payment) constituted the amount of the consideration to be included in the transaction price and has been allocated to the single performance obligation. The basis for the conclusions regarding the treatment of development and sales-based milestones associated with the Second Target are consistent with those associated with the initial combined performance obligation under the BI Agreement. The Company will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur. The $5.0 million transaction price, of which no revenue was recognized in 2018 as the program had not yet commenced, will be recognized over the research term, which is currently estimated to extend through June 2022.

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In addition to establishing the terms of the Second Target option exercise, the ATA also amends the BI Agreement to provide BI with the option to add, over a three-year period, the development of product candidates targeting a further additional target gene (the "Third Target Option").

Per the ATA, if BI elects, in its sole discretion, to exercise the Third Target Option, the parties would agree to a research work plan and budget for the additional gene and negotiate development and commercialization milestones and royalty payments to the Company, and BI would make an option fee payment to the Company of $5.0 million. This option exercise fee is consistent with the Second Target option exercise fee, which management concluded was representative of the standalone selling price. If BI chooses to exercise the Third Target option, the Company will be responsible for the discovery and initial profiling of the product candidates, including primary preclinical studies, synthesis, and delivery. BI will be responsible for evaluating and selecting the product candidates for further development. If BI selects one or more product candidates, it will be responsible for further preclinical development, clinical development, manufacturing, and commercialization of those products. If the Third Target Option is exercised, such exercise would result in a new contract for accounting purposes, as the licensing rights and research and development services underlying the Third Target Option are distinct from those associated with the initial and Second Target.

During the year ended December 31, 2018, the Company recognized $6.1 million of revenue related to the BI Agreement, as amended, in the accompanying consolidated statement of operations. The aggregate amount of the transaction price allocated to the Company's partially unsatisfied performance obligations and recorded as deferred revenue at December 31, 2018 is $3.2 million, all of which is included in current portion of deferred revenue. The Company expected to recognize this amount over the remaining research program term, which is eight months as of December 31, 2018.

The following table presents changes in the Company's aggregate deferred revenue balances for each reporting period:

	YEAR ENDED DECEMBER 31, 2018			
	BALANCE AT BEGINNING OF PERIOD	ADDITIONS	DEDUCTIONS	BALANCE AT END OF PERIOD
Deferred revenue, current and noncurrent	$9,270	$ 180,092	$ (6,176)	$ 183,186
	YEAR ENDED DECEMBER 31, 2017			
	BALANCE AT BEGINNING OF PERIOD	ADDITIONS	DEDUCTIONS	BALANCE AT END OF PERIOD
Deferred revenue, current and noncurrent	$—	$ 10,300	$ (1,030)	$ 9,270

The Company had no deferred revenue during the year ended December 31, 2016.

9. STOCKHOLDERS' EQUITY

Preferred stock

The Company has authorized up to 5,000,000 shares of preferred stock, $0.0001 par value per share, for issuance. The preferred stock will have such rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, as shall be determined by the Company's board of directors upon its issuance. At December 31, 2018 and 2017, there were no shares of preferred stock outstanding.

As further disclosed in Note 10, during the year ended December 31, 2017, the Company issued and sold in a private placement 700,000 shares of its newly designated redeemable convertible preferred stock, par value $0.0001 per share. Redeemable convertible preferred shares and the shares of common stock issuable upon conversion of the redeemable convertible preferred stock were offered and sold by the Company pursuant to an exemption from the

registration requirements of the Securities Act provided by Section 4(a)(2) thereunder. On December 18, 2017, the Company completed the conversion of the redeemable convertible preferred stock and issued an aggregate of 24,206,663 shares of the Company's common stock.

Issuances of Common Stock

On December 18, 2017, the Company completed an underwritten follow-on public offering of 5,714,286 shares of common stock (the "2017 Offering"), which was made pursuant to the Company's effective registration statement on Form S-3 previously filed with the SEC. In connection with the 2017 Offering, the Company entered into an underwriting agreement (the "2017 Underwriting Agreement") with Stifel, Nicolaus & Company, Incorporated and Evercore Group LLC as representatives of the underwriters listed in the 2017 Underwriting Agreement (collectively, the "2017 Underwriters"), pursuant to which the Company granted to the 2017

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Underwriters a 30-day option to purchase up to an additional 857,143 shares of the Company's common stock (the "Overallotment"). The Company completed the sale of 6,571,428 shares, inclusive of the Overallotment, to the 2017 Underwriters on December 18, 2017, and that sale resulted in the receipt by the Company of aggregate gross proceeds of $46.0 million, less underwriter commissions and additional offering expenses totaling approximately $3.2 million.

On April 20, 2018, the Company entered into a Share Issuance Agreement with Alnylam ("Alnylam Share Issuance Agreement"), pursuant to which the Company agreed to issue to Alnylam 983,208 shares in satisfaction of the Company's obligation under the Settlement Agreement to deliver shares to Alnylam (see Note 15). The Alnylam Share Issuance Agreement contains customary representations and warranties of each party. The transaction contemplated by the Alnylam Share Issuance Agreement was closed on April 24, 2018.

On September 11, 2018, the Company completed an underwritten follow-on public offering of 7,680,492 shares of common stock (the "2018 Offering"). In connection with the 2018 Offering, the Company entered into an underwriting agreement (the "2018 Underwriting Agreement") with Citigroup Global Markets Inc. and Leerink Partners LLC as representatives of the underwriters listed in the 2018 Underwriting Agreement (collectively, the "2018 Underwriters"), pursuant to which the Company granted to the 2018 Underwriters a 30-day option to purchase up to an additional 1,152,073 shares of the Company's common stock. The Company completed the sale of 8,832,565 shares to the 2018 Underwriters on September 11, 2018; the sale resulted in the receipt of gross proceeds of $115.0 million.

In connection with the Alexion Collaboration Agreement, the Company and Alexion entered into the Alexion Share Issuance Agreement on October 22, 2018, pursuant to which the Company sold to Alexion 835,834 shares of the Company's common stock at $17.95 per share for an aggregate stated purchase price of approximately $15.0 million, of which $9.1 million was allocated to the share issuance for accounting purposes. Pursuant to the terms of the Alexion Share Issuance Agreement, Alexion may not, without the prior approval of the Company, dispose of any of the Alexion shares for a six-month period of time commencing on the closing date of the Alexion Share issuance.

In connection with the Lilly Collaboration Agreement, the Company and Lilly entered into the Lilly Share Issuance Agreement on October 25, 2018, pursuant to which the Company sold to Lilly 5,414,185 shares of common stock at $18.47 per share for an aggregate stated purchase price of approximately $100.0 million, of which $51.3 million was allocated to the share issuance for accounting purposes. The closing of the transactions contemplated by the Lilly Collaboration Agreement and the Lilly Share Issuance Agreement occurred on December 19, 2018.

10. REDEEMABLE CONVERTIBLE PREFERRED STOCK

On April 11, 2017, pursuant to a redeemable convertible preferred stock purchase agreement ("SPA") with seven institutional investors (the "Preferred Holders"), led by funds advised by Bain Capital Life Sciences L.P. ("Lead Investor"), the Company issued and sold in a private placement 700,000 shares of its newly designated redeemable convertible preferred stock, par value $0.0001 per share, at a purchase price of $100.00 per share, for total gross proceeds of $70.0 million ("Private Placement"), less issuance costs of approximately $0.8 million. The redeemable convertible preferred shares and the shares of common stock issuable upon conversion of the redeemable convertible preferred stock were offered and sold by the Company pursuant to an exemption from the registration requirements of the Securities Act provided by Section 4(a)(2) thereunder.

In addition to the Lead Investor, other participants in the Private Placement included affiliates of Cormorant Asset Management, LLC, Domain Associates, LLC ("Domain Associates"), EcoR1 Capital, LLC, RA Capital Management, LLC ("RA Capital") and Skyline Management LLC ("Skyline Ventures"), among others. Domain Associates, RA Capital and Skyline Ventures are entities that are affiliated or were formerly affiliated with certain members of the Company's board of directors. On March 28, 2017, in accordance with the terms of the SPA, the Company increased the size of its board of directors from eight to nine directors and approved the appointment of Adam M. Koppel, M.D., Ph.D., a managing director of the Lead Investor, as a director of the Company, effective as of the closing of the Private Placement on April 11, 2017. Dr. Koppel was reelected to the Company's board of directors by shareholder vote in June 2017.

The redeemable convertible preferred stock had the rights and preferences set forth in a Certificate of Designation, which was filed with the Secretary of State of the State of Delaware.

Inducement and conversion

On December 13, 2017, in connection with the 2017 Offering, defined and discussed in Note 9, the Company entered into a letter agreement (the “Letter Agreement”) with the Preferred Holders. Pursuant to the Letter Agreement, the Preferred Holders agreed, subject to the completion of the 2017 Offering, to optionally convert all of their shares of redeemable convertible preferred stock, to the extent not subject to Conversion Blockers, into common stock, and consented, where applicable, to the repurchase of the residual

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shares of common stock that would have been issuable but for the Conversion Blockers (the "Residual Shares") for $0.0001 per share. "Conversion Blockers" refers to the beneficial ownership limitations in the Company's Certificate of Designation of the redeemable convertible preferred stock, which included (i) a 19.99% blocker provision to comply with Nasdaq Listing Rules, (ii) if so elected by a holder, a 9.99% blocker provision that would have prohibited beneficial ownership of more than 9.99% of the outstanding shares of the Company's common stock or voting power at any time, and (iii) ownership limitations resulting from applicable regulatory restrictions.

The Letter Agreement also provided for Preferred Holders to waive and amend certain provisions in an amended and restated registration rights agreement by and among the Company and the Preferred Holders party thereto (the "Registration Rights Agreement"). In consideration for the Preferred Holders' agreeing to the optional conversion of the redeemable convertible preferred stock and to a waiver under and certain amendments to the Registration Rights Agreement, the Company agreed to issue to the Preferred Holders pre-funded warrants (the "Pre-Funded Warrants"), exercisable in part or in whole at any time upon grant for shares of the Company's common stock at a price per share of $0.0001 per share. Each Preferred Holder was entitled to elect to receive shares of the Company's common stock in lieu of the Pre-Funded Warrants that otherwise would have been issued to such Preferred Holder subject to any applicable Conversion Blockers. Under the Letter Agreement, the number of shares allocable to each Preferred Holder was calculated based on the sum of (i) the number of shares of common stock into which the additional dividend accruals on the redeemable convertible preferred stock that such Preferred Holders would have been entitled to receive up to and including March 31, 2018 would have been convertible, calculated immediately prior to the effectiveness of the conversion and (ii) any Residual Shares repurchased, or to be repurchased, from such Preferred Holder by the Company as described above (collectively, the "Additional Investor Shares"). The formula for the Additional Investor Shares assumes (1) a conversion price of $3.19 per share of common stock; (2) application of a dividend rate of 12% per annum from April 11, 2017 to October 27, 2017 and (3) application of a dividend rate of 8% per annum commencing from October 28, 2017 through March 31, 2018.

On December 18, 2017, the Company completed the conversion of the redeemable convertible preferred stock and issued an aggregate of 24,206,663 shares of the Company's common stock. No Pre-Funded Warrants were issued in connection with the conversion of the redeemable convertible preferred stock, as all Preferred Holders opted to receive common shares in lieu of Pre-Funded Warrants, largely given the inapplicability of Conversion Blockers as of the date of conversion, immediately after which no shares of redeemable convertible preferred stock remained outstanding.

On December 29, 2017, the Company filed with the Secretary of State of the State of Delaware a Certificate of Elimination of the Redeemable Convertible Preferred Stock, which eliminates from the Company's Certificate of Incorporation all matters set forth in the Certificate of Designation of Redeemable Convertible Preferred Stock previously filed with the Secretary of State of the State of Delaware, which established and designated the redeemable convertible preferred stock and the rights, powers, preferences, privileges and limitations thereof.

Upon conversion of the redeemable convertible preferred stock, the Company applied the guidance outlined in the FASB's Accounting Standard Codification ("ASC") Topic 470-20, Debt with Conversion and Other Options ("ASC 470-20"), which contains guidance addressing the accounting for induced conversions of convertible debt, which in turn, per the U.S. Securities and Exchange Commission's ("SEC") guidance codified in ASC Topic 260, Earnings per Share ("ASC 260"), should be applied also to induced conversions of convertible preferred stock.

The Company applied the guidance provided in ASC 260-10-S99-2 and compared the fair value of common stock transferred in the conversion transaction to the Preferred Holders to the fair value of common stock issuable pursuant to the original conversion terms. The resulting excess, which amounted to approximately $3.8 million, was recorded as a deemed dividend on conversion of the redeemable convertible preferred shares and has been added to net loss to arrive at net loss attributable to common stockholders in the accompanying consolidated statement of operations for the year ended December 31, 2017.

Dividends

Each holder of redeemable convertible preferred stock had been entitled to receive cumulative dividends on the Accrued Value, as defined below, of each share of redeemable convertible preferred stock at an initial rate of 12% per annum, compounded quarterly and subject to two rate reductions of 4% each in connection with the occurrence of one

of the agreed-upon milestone events. Entering into the BI Agreement, as defined and discussed in Note 8, constituted, per the Certificate of Designation, a milestone event for purposes of applying the first of two allowable rate reductions to dividends payable on the redeemable convertible preferred stock. As such, the dividend rate on the redeemable convertible preferred stock was reduced from 12% to 8%, effective on October 27, 2017. Dividends on the redeemable convertible preferred stock accrued on the Accrued Value of each share of redeemable convertible preferred until the conversion thereof, which occurred on December 18, 2017, as discussed above. “Accrued Value” meant, with respect to each share of redeemable convertible preferred stock, the sum of (i) $100.00 plus (ii) on each quarterly dividend date, an additional amount equal to the dollar value of any dividends on a share of redeemable convertible preferred stock which had accrued on any dividend payment date and had not previously been added to such Accrued Value.

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For accounting purposes, in accordance with ASC Topic 480-10-S99, Distinguishing Liabilities from Equity – SEC Materials ("ASC 480-10-S99"), the Company recorded the dividends at fair value at each dividend declaration date. The fair value of the dividends was determined using a binary lattice model that captured the intrinsic value of the underlying common stock on the declaration date and the option value of the shares and future dividends.

The lattice model was used to determine fair value of dividends on each dividend date through September 30, 2017, which was the last dividend date prior to conversion of the redeemable convertible preferred shares, included the following inputs:

	JUNE 30, 2017	SEPTEMBER 30, 2017
Price per common share	$3.17	$ 5.75
Expected term (in years)	6.75	6.50
Expected volatility	70.0 %	73.0 %
Risk-adjusted discount rate	18.0 %	19.1 %

In addition to the inputs presented above, use of the lattice model applied other assumptions, including probability simulations of various outcomes largely associated with the conversion-related milestone events referred to above and with the progression of the Company's per common share price. Use of the lattice model resulted in a fair value estimate of the aggregate dividends declared on June 30, 2017 and September 30, 2017 of $1.9 million and $4.1 million, respectively.

Beneficial conversion feature

In accordance with ASC Topic 470-20, the Company recorded a beneficial conversion feature ("BCF") related to the issuance of the redeemable convertible preferred. The BCF was recognized separately at issuance by allocating a portion of the proceeds equal to the intrinsic value of that feature to additional paid-in capital. The BCF was calculated at the commitment date, which management has determined to be the date of issuance. Intrinsic value is calculated as the difference between the effective conversion price and the fair value of the Company's common stock, multiplied by the number of shares into which the issued shares of redeemable convertible preferred shares are convertible. During the year ended December 31, 2017, the Company recorded a deemed dividend charge of $6.1 million, to reflect full and immediate accretion of the discount resulting from the at-issuance BCF embedded within the redeemable convertible preferred stock as a result of the shares being immediately convertible into shares of the Company's common stock at the option of the Preferred Holders.

Accretion of the discount resulting from the BCF and cumulative dividends, including accretion of share issuance costs, were non-cash transactions and have been reflected below net loss to arrive at net loss attributable to common stockholders.

The following table reflects the changes in redeemable convertible preferred shares recorded during the year ended December 31, 2017:

Balance at January 1, 2017	$ —
Issuance of redeemable convertible preferred shares	70,000
Share issuance costs	(750)
Net proceeds	69,250
Discount resulting from the BCF at issuance	(6,144)
Accretion of the discount resulting from the BCF (deemed dividend)	6,144
Dividends accrued at the stated rates	5,515
Fair value in excess of dividends accrued at the stated rates	3,846
Accretion of share issuance costs (additional dividends)	750
Balance immediately prior to conversion	79,361
Conversion of redeemable convertible preferred shares	(79,361)
Balance at December 31, 2017	$ —

11. STOCK-BASED COMPENSATION

Equity Incentive Plans

As of December 31, 2018, the Company's approved equity incentive plans include: the Third Amended and Restated 2007 Employee, Director and Consultant Stock Plan ("2007 Plan"); the 2010 Employee, Director and Consultant Equity Incentive Plan ("2010 Plan");

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2014 Employee Stock Purchase Plan ("2014 ESPP"); Amended and Restated 2014 Performance Incentive Plan ("2014 Plan"); and, the 2016 Inducement Plan ("2016 Plan"). These plans are administered by the board of directors and permit the granting of stock options, stock appreciation rights, stock bonuses, restricted stock, performance stock, stock units, phantom stock or similar rights to purchase or acquire shares. Upon adoption of the 2014 Plan, the Company no longer grants new equity awards under its 2007 Plan or 2010 Plan.

Amended and Restated 2014 Performance Incentive Plan

On January 14, 2014, the board of directors adopted 2014 Plan which authorized the issuance of up to 1,900,000 shares of the Company's common stock, with an additional increase on the first trading day in January of each calendar year during the term of the plan by an amount equal to 4% of the total number of shares of Common Stock issued and outstanding on December 31 of the immediately preceding calendar year. In June 2015, the 2014 Plan was amended to increase the replenishment percentage from 4% to 5% of outstanding common shares annually and to allow the reissuance thereunder of awards and grants that expire or are canceled, terminated, forfeited or fail to vest under the 2007 Plan and 2010 Plan, as amended. Stock options for new hires granted under this plan generally vest 25% after 12 months, followed by ratable vesting over the remaining 36-month term and expire 10 years from the grant date. Annual promotional and incentive-related grants generally vest ratably over a period of 48 months.

As of December 31, 2018, there were 872,411 shares of common stock reserved for future issuance under the 2014 Plan.

Inducement Grants

During 2014 and 2015, the Company granted 470,272 and 450,700 stock options, respectively, as an inducement material to individuals entering into employment with the Company ("Inducement Grants"). The Inducement Grants were approved by the Compensation Committee of the Company's board of directors and were awarded in accordance with Nasdaq Listing Rule 5635(c)(4) and outside of the 2014 Plan. As such, any shares underlying the Inducement Grants are not, upon forfeiture, cancellation or expiration, returned to a pool of shares reserved for future issuance. As of December 31, 2018, there were 130,000 Inducement Grants that remained outstanding.

2016 Inducement Plan

On March 4, 2016, the board of directors adopted the 2016 Plan pursuant to which the Company may grant options to purchase common shares as an inducement to individuals to join the Company. The 2016 Plan, as adopted, allowed the Company to deliver up to 250,000 shares (the "Share Limit") of its common stock to eligible persons, as defined. The Share Limit is subject to adjustment as contemplated by the provisions of the 2014 Plan. In February and May 2017, the Share Limit was adjusted to increase the pool of issuable options by 125,000 and 200,000 underlying shares, respectively. On December 11, 2018, the board of directors approved a resolution to further increase the Share Limit under the 2016 Plan by 2,700,000 to 3,275,000 underlying shares. There were no stock options granted pursuant to the 2016 Plan during the year ended December 31, 2018.

As of December 31, 2018, the Company has 2,875,000 shares of common stock reserved for future issuance under the 2016 Plan.

Stock-based compensation expense

The Company has classified stock-based compensation expense in its consolidated statements of operations as follows:

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
Research and development expenses	$3,062	$3,536	$4,467
General and administrative expenses	4,826	4,234	4,698
Total	$7,888	$7,770	$9,165

Stock options

Expected volatility for the Company's common stock was determined based on an average of the historical volatility of a peer group of similar companies due to limited historical volatility of the Company's own common stock. The Company also has limited stock option exercise information, and as such, the expected term of stock options granted was calculated in most cases using the simplified method, which represents the average of the contractual term of the

stock option and the weighted average vesting period of the stock option. The assumed dividend yield is based upon the Company's expectation of not paying dividends in the foreseeable future. The

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risk-free rate for periods within the expected life of the stock option is based upon the U.S. Treasury yield curve in effect at the time of grant.

The assumptions used in the Black-Scholes option-pricing model for all stock options granted during each period presented are as follows:

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
Common stock price	$9.14 - $15.74	$2.49 – $9.71	$2.94 – $9.09
Expected option term (in years)	5.50 - 6.25	5.50 – 6.25	5.50 – 6.25
Expected volatility	75.9% - 78.3%	79.4% – 91.1%	70.9% – 79.4%
Risk-free interest rate	2.3% - 3.0%	1.9% – 2.2%	1.2% – 2.0%
Expected dividend yield	0.0%	0.0%	0.0%

The table below summarizes the activity under the Company's equity incentive plans:

	NUMBER OF OPTIONS	WEIGHTED-AVERAGE EXERCISE PRICE	WEIGHTED-AVERAGE REMAINING CONTRACTUAL TERM (YEARS)	AGGREGATE INTRINSIC VALUE
OUTSTANDING – JANUARY 1, 2018	6,124,096	$ 8.58		
Granted	2,241,350	$ 11.07		
Exercised	(329,934)	$ 5.31		
Forfeited/Canceled	(90,026)	$ 5.71		
Expired	(157,796)	$ 14.08		
OUTSTANDING – DECEMBER 31, 2018	7,787,690	$ 9.36	7.3	$ 24,305
EXERCISABLE – DECEMBER 31, 2018	4,842,084	$ 9.71	6.4	$ 16,151
VESTED AND EXPECTED TO VEST – DECEMBER 31, 2018	7,567,801	$ 9.30	7.2	$ 23,948

The weighted average grant date fair value of stock options granted during the years ended December 31, 2018, 2017, and 2016 was $7.64, $2.52, and $4.60 per share, respectively. As of December 31, 2018, there was $17.1 million of unrecognized compensation cost related to unvested employee stock options which are expected to be recognized over a weighted average period of 2.9 years. The intrinsic value of stock options exercised was $2.9 million, $0.1 million, and $0.2 million for the years ended December 31, 2018, 2017, and 2016, respectively.

The Company does not currently hold any treasury shares. Upon stock option exercise, the Company issues new shares and delivers them to the participant.

Restricted common stock

In 2014, the Company issued a total of 44,000 shares of the Company's restricted common stock, of which 4,000 shares were fully vested at the grant date and the remaining shares were scheduled to vest in equal tranches over a four-year period on the anniversary date of the related grant. The fair value of these shares totaled $0.7 million at the grant date, representing a weighted average grant date fair value per share of $16.30.

At December 31, 2017, there were 10,000 shares of the Company's restricted common stock remaining outstanding with a weighted average grant date fair value of $16.30. During the year ended December 31, 2018, all 10,000 shares of restricted common stock with a weighted average grant date fair value of $16.30 vested and there are no outstanding shares of restricted common stock at December 31, 2018. The total fair value of restricted common stock vested during the years ended December 31, 2018 and 2016 was $0.1 million and $0.1 million, respectively. The total fair value of restricted common stock that vested during the year ended December 31, 2017 was immaterial.

Common stock warrants

At December 31, 2017, the Company had 87,901 common stock warrants outstanding with a weighted average exercise price of $13.80. All of the Company's outstanding common stock warrants have been exercisable since November 30, 2013.

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During the year ended December 31, 2018, certain warrant holders exercised warrants to purchase 85,703 shares of the Company's common stock on a net basis and received 45,710 shares of common stock and 39,993 shares were used to cover the exercise price of $7.00 per share. At December 31, 2018, there were 2,198 common stock warrants remaining outstanding with an exercise price of $250.00 per share and a remaining contractual life of 1.46 years.

Employee stock purchase plan

On January 28, 2014, the Company's stockholders approved the 2014 ESPP, which authorized the issuance of up to 1,000,000 shares of common stock thereunder. The 2014 ESPP provides for an automatic reserve increase equivalent to the lesser of 1% of the total number of shares of common stock issued and outstanding on December 31 of the immediately preceding calendar year and 1,000,000 shares of common stock, unless otherwise determined by the Company's board of directors. As of December 31, 2018, there were 2,107,791 shares of common stock authorized and 1,845,179 shares of common stock available for issuance under the 2014 ESPP.

Eligible employees may purchase shares of the Company's common stock through regular payroll deductions up to 15% of their eligible compensation. Under the terms of the offering under the 2014 ESPP, the number of shares purchased by an individual participant in the plan may not exceed 10,000 shares in any one purchase period. In addition, the fair market value of shares purchased by an individual participant in the plan may not exceed $25,000 if the contribution period is within any one calendar year. Participants are allowed to terminate their participation in the ESPP at any time during the purchase period prior to the purchase of the shares. The offering periods have a 24 month term; which consists of four purchase periods, each of which is six months in duration. New offering periods commence on the first day of January and July each year and end on the last business day of the immediately following June or December, respectively.

The per-share purchase price at the end of each offering period is equal to the lesser of 85% of the fair market value of the common stock on the grant date of the offering period to which the purchase period relates or 85% of the fair market value of the common stock on the purchase date of the applicable purchase period. In the event that the fair value of the common stock on any purchase date during an offering period is lower than the fair market value of the common stock on the grant date of that offering period, that offering period will terminate on such purchase date, and each participant in such terminated offering period will be automatically enrolled in the new offering period that commences on the first business day of the next offering period that immediately follows such purchase date.

Shares issued under the 2014 ESPP are considered compensatory. Accordingly, the Company is required to measure the fair value of the stock purchase rights granted and record compensation expense for share purchase rights granted under the 2014 ESPP. The fair values of the stock purchase rights are estimated using the Black-Scholes option-pricing model, which relies on a number of key assumptions to in calculating the estimates of fair value. Stock-based compensation expense related to stock purchase rights under the 2014 ESPP was $0.1 million, $0.4 million, $0.4 million and for the years ended December 31, 2018, 2017, and 2016.

During the years ended December 31, 2018, 2017, and 2016, the Company issued 118,239, 84,890, and 36,501 shares of common stock under the 2014 ESPP, respectively. The weighted average purchase price of shares issued under the 2014 ESPP were $2.61, $2.45, and $4.53 per share for the years ended December 31, 2018, 2017, and 2016, respectively.

12. 401(K) PROFIT SHARING PLAN AND TRUST

The Company has a 401(k) Profit Sharing Plan and Trust ("401(k) Plan"), which is a retirement plan in which substantially all employees are eligible to participate. Eligible employees may elect to contribute up to the maximum limits, as set by the Internal Revenue Service, of their eligible compensation. Under the terms of the 401(k) Plan, employees may elect to make pre-tax and Roth contributions through payroll deductions within statutory and plan limits. The Company makes matching contributions of 300% of eligible employee salary deferrals that do not exceed 2% of the eligible participant's compensation. All matching contributions vest immediately. Each year, the Company may also make a discretionary profit sharing contribution to the plan. Such contributions to the Plan are allocated among eligible participants in the proportion of their salaries to the total salaries of all participants.

Expense recognized by the Company for matching contributions made to the 401(k) Plan was $0.6 million, $0.4 million, and $0.2 million for the years ended December 31, 2018, 2017, and 2016, respectively. There were no discretionary profit sharing contributions made by the Company during the years ended December 31, 2018, 2017, or

2016.

13. INCOME TAXES

The Company has no current and no deferred income tax expense for the years ended December 31, 2018 and 2017, respectively. The Company did not record a federal income tax provision or benefit for the years ended December 31, 2018, 2017, and 2016, respectively.

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The reconciliation between income taxes computed at the federal statutory income tax rate and the provision for (benefit from) income taxes is as follows:

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
Federal statutory rate	21.0 %	34.0 %	34.0 %
Effect of:			
Foreign rate differential	(9.5)%	(17.6)%	(31.4)%
Tax reform	— %	(29.6)%	— %
Net operating loss limitation	(23.0)%	— %	— %
Change in valuation allowance	10.6 %	13.5 %	— %
Research and development tax credit	0.5 %	0.6 %	(0.7)%
Stock-based compensation expense	0.4 %	(0.8)%	(0.9)%
Other	— %	(0.1)%	(1.0)%
Total	— %	— %	— %

The components of the Company's deferred tax assets are as follows:

	DECEMBER 31,	
	2018	2017
Deferred tax assets:		
Net operating loss carryforwards	$24,739	$32,008
Capitalized research and development costs	516	618
Research and development credit carryforwards	3,988	3,481
Stock-based compensation expense	7,644	6,066
Depreciation expense and other costs	75	42
Net deferred tax assets	36,962	42,215
Valuation allowance	(36,962)	(42,215)
Net deferred tax assets	$—	$—

On December 22, 2017, the Tax Cuts and Jobs Act (the "TCJA") was signed into law in the United States. The TCJA reduced the U.S. corporate tax rate from 34% to 21% for tax years beginning after December 31, 2017. As a result of the newly enacted law, the Company was required to revalue all deferred tax assets and liabilities existing as of December 31, 2017 so as to reflect the reduction in the federal tax rate. This revaluation resulted in a reduction to the Company's deferred tax asset of $17.8 million at December 31, 2017, with a corresponding reduction to the Company's valuation allowance. Consequently, there was no impact on the accompanying consolidated financial statements that resulted from the reduction in the federal tax rate. Other relevant provisions of the TCJA did not have a material impact on the accompanying consolidated financial statements.

Management has evaluated the positive and negative evidence bearing upon the realizability of the Company's net deferred tax assets and has determined that it is more likely than not that the Company will not recognize the benefits of the net deferred tax assets. As a result, the Company has recorded a full valuation allowance at December 31, 2018 and 2017.

Realization of the future tax benefits is dependent on many factors, including the Company's ability to generate taxable income within the net operating loss carryforward period. Under the provisions of the Internal Revenue Code, certain substantial changes in the Company's ownership, including a sale of the Company or significant changes in ownership due to sales of equity, may have limited, or may limit in the future, the amount of net operating loss carryforwards which could be used annually to offset future taxable income. At this time, there is an estimated limitation of approximately $97.0 million of net operating losses.

As of December 31, 2018, the Company had approximately $158.7 million of federal and $148.2 million of state net operating loss carryforwards. If not utilized, the federal and state net operating loss carryforwards expire starting in 2028 and 2030, respectively. Additionally, as of December 31, 2018, the Company had $2.6 million of federal and $1.4 million of Massachusetts tax credits that expire starting in 2028 and 2023, respectively.

As of December 31, 2018, the Company had $1.6 million of unrecognized tax benefits, all of which would affect income tax expense if recognized, before consideration of the Company's valuation allowance. The Company does not expect the unrecognized tax benefits to change significantly over the next 12 months. The Company recognizes both interest and penalties associated with

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uncertain tax positions as a component of income tax expense. As of December 31, 2018 and 2017, the Company had no accrued penalties or provisions for interest.

A reconciliation of the gross unrecognized tax benefit is as follows:

	YEAR ENDED DECEMBER 31,	
	2018	2017
Unrecognized tax benefits at the beginning of the period	$1,451	$1,210
Additions for current tax positions	211	243
Changes for previous tax positions	(31)	(2)
Unrecognized tax benefits at the end of the period	$1,631	$1,451

The Company files income tax returns in the United States, the Commonwealth of Massachusetts, Colorado, and New Jersey. The tax years 2008 through 2017 remain open to examination by these jurisdictions, as carryforward attributes generated in past years may be adjusted in a future period. The Company is not currently under examination by the Internal Revenue Service or any other jurisdiction for these years. The Company has not recorded any interest or penalties for unrecognized tax benefits since its inception.

14. COMMITMENTS AND CONTINGENCIES

Facility lease

On July 11, 2014, the Company executed a non-cancelable operating lease for office and laboratory space in Cambridge, Massachusetts. The lease agreement, the term of which commenced on December 1, 2014, obligates the Company to make minimum payments totaling $9.6 million over a six-year lease term ending November 30, 2020. The Company has the option to extend the lease term for one additional five-year period. Rent expense is recorded on a straight-line basis.

As part of the lease agreement, the Company established a letter of credit, secured by a restricted money market account, the balance of which is presented as restricted cash equivalents at December 31, 2018 and 2017.

Future minimum lease payments on the Company's non-cancelable operating lease for office and laboratory space are as follows:

YEARS ENDING DECEMBER 31,	OPERATING LEASE
2019	$ 1,678
2020	1,581
Total	$ 3,259

Rent expense was $1.6 million for each of the years ended December 31, 2018, 2017, and 2016.

Legal proceedings

From time to time, the Company may be subject to various claims and legal proceedings. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount is reasonably estimable, the Company will accrue a liability for the estimated loss. There were no contingent liabilities recorded as of December 31, 2018 or 2017.

15. LITIGATION

On June 10, 2015, Alnylam filed a complaint against the Company in the Superior Court of Middlesex County, Massachusetts. The complaint alleged misappropriation of confidential, proprietary, and trade secret information, as well as other related claims, in connection with the Company's hiring of a number of former employees of Merck & Co., Inc. ("Merck") and its discussions with Merck regarding the acquisition of its subsidiary, Sirna Therapeutics, Inc., which was subsequently acquired by Alnylam.

On April 18, 2018, the Company and Alnylam entered into the Settlement Agreement, resolving all ongoing litigation between the Company and Alnylam. The terms of the Settlement Agreement include mutual releases and dismissals with prejudice of all claims and counterclaims in the following litigation between the parties: (i) Alnylam Pharmaceuticals, Inc. v. Dicerna Pharmaceuticals, Inc., No. 15-4126 pending in the Massachusetts Superior Court for Middlesex County and (ii) Dicerna Pharmaceuticals, Inc., v. Alnylam Pharmaceuticals, Inc. No.1:17-cv-11466 pending in the United States District Court for the District of Massachusetts. Pursuant to the terms of the Settlement

Agreement, the Company has agreed to make the following payments to Alnylam: (i) a $2.0 million upfront payment in cash, which the Company made in May 2018; (ii) an additional $13.0 million in cash to be paid as 10.0% of any upfront or

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first year cash consideration that the Company receives pursuant to future collaborations related to Ga1NAc-conjugated RNAi research and development (excluding any amounts received or to be received by the Company from its existing collaboration with BI), provided that the $13.0 million must be paid by no later than April 28, 2022; and (iii) issuance of shares of the Company's common stock pursuant to the Alnylam Share Issuance Agreement.

Under the Settlement Agreement, for periods ranging from 18 months up to four years, the Company will be restricted in its development and other activities relating to oligonucleotide-based therapeutics directed toward a defined set of eight Alnylam targets (the "Oligo Restrictions"). The Oligo Restrictions pertain to targets where Dicerna does not have, or does not currently intend to have, a therapeutic program, or are expected to be consistent with Dicerna's execution on programs in the normal course of business. The Settlement Agreement did not include any admission of liability or wrongdoing by either party or any licenses to any intellectual property from either party.

On April 20, 2018, the Company and Alnylam entered into the Alnylam Share Issuance Agreement, pursuant to which the Company agreed to issue to Alnylam 983,208 shares in satisfaction of the Company's obligation under the Settlement Agreement to deliver shares to Alnylam. The 983,208 shares issued pursuant to the Alnylam Share Issuance Agreement was recorded at fair market value of $10.3 million based on the Company's closing share price on April 18, 2018, the date the Settlement Agreement was executed. The Company did not assign any value to the Oligo Restrictions as the Company did not incur additional losses or give up any value as a result of the restrictions.

In May 2018, the Company recorded the cash obligation of $13.0 million as a liability discounted to the estimated present value of $8.7 million at an effective interest rate of 10.0%. The Company applied the effective interest method, as the present value is accreted through maturity. In October 2018, the Company entered into collaboration agreements with Alexion and Lilly, under which the Company is entitled to upfront cash consideration of $22.0 million and $100.0 million, respectively (see Note 8). Accordingly, the Company revised its estimate of the present value of the litigation settlement payable from $8.7 million to $13.0 million based on the expected timing of the remaining payments. The impact of revising the expected timing of repayment was recorded as a $3.7 million charge to litigation expense in the consolidated statement of operations for the year ended December 31, 2018.

In connection with the execution of the Alexion Collaboration Agreement and related the receipt the non-refundable, non-reimbursable, and non-creditable upfront payment of $22.0 million and proceeds of $15.0 million from the Alexion Share Issuance Agreement in October 2018, the Company determined that $2.5 million became payable to Alnylam under the terms of the Settlement Agreement. The Company issued a payment to Alnylam of $2.5 million in November 2018 for the amount of the litigation settlement payable due in connection with the cash consideration received from Alexion during 2018.

At December 31, 2018, the outstanding balance of the litigation settlement payable was $10.5 million. The Company paid the remaining outstanding balance of litigation settlement payable in full on January 22, 2019. During the year ended December 31, 2018, the Company recognized interest expense of $0.6 million on the outstanding balance of the litigation settlement payable during the year.

Total litigation expense was $29.1 million for the year ended December 31, 2018, all of which related to the litigation and settlement agreement with Alnylam. The litigation expense for the year ended December 31, 2018 includes $24.7 million related to the Settlement Agreement. The Company recorded expenses related to the Alnylam litigation of $9.0 million during the year ended December 31, 2017.

16. QUARTERLY FINANCIAL DATA (UNAUDITED)

The following tables contain selected quarterly financial information for the years ended December 31, 2018 and 2017. The Company believes that the following information reflects all normal recurring adjustments necessary for a fair statement of the information for the periods presented.

	2018				
	FIRST QUARTER	SECOND QUARTER	THIRD QUARTER	FOURTH QUARTER	TOTAL YEAR
Revenue from collaborative arrangements	$1,545	$1,545	$1,545	$1,541	$6,176
Net loss	$(15,579)	$(35,644)	$(19,020)	$(18,610)	$(88,853)
Net loss attributable to common stockholders	$(15,579)	$(35,644)	$(19,020)	$(18,610)	$(88,853)

Net loss per share attributable to common stockholders – basic and diluted	$(0.30)	$(0.68)	$(0.35)	$(0.29)	$(1.60)

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	2017				
	FIRST QUARTER	SECOND QUARTER	THIRD QUARTER	FOURTH QUARTER	TOTAL YEAR
Revenue from collaborative arrangements	$—	$—	$—	$1,030	$1,030
Net loss	$(14,201)	$(15,225)	$(15,033)	$(15,741)	$(60,200)
Net loss attributable to common stockholders	$(14,201)	$(23,991)	$(19,144)	$(22,956)	$(80,292)
Net loss per share attributable to common stockholders – basic and diluted	$(0.68)	$(1.15)	$(0.92)	$(0.91)	$(3.66)

Net loss per share attributable to common stockholders is based on each reporting period's weighted average number of shares outstanding, which may differ on a quarter-to-quarter basis. As such, the sum of the quarterly net loss per share attributable to common stockholders may not equal the year-to-date net loss per share attributable to common stockholders.

17. SUBSEQUENT EVENTS

Lexington lease

On January 2, 2019, the Company entered into a non-cancelable real property lease agreement with Hayden Office Trust under a Declaration of Trust dated August 24, 1977, as the same may have been amended, for approximately 80,872 square feet of laboratory and office space in Lexington, Massachusetts (the "Lexington Lease"). The Company intends to move its corporate headquarters and research facility to this location upon occupancy, which is expected to occur in the fourth quarter of 2019.

The original term (the "Original Term") of the Lexington Lease is seven years, commencing on the earlier of (a) the date on which the premises are ready for occupancy under the terms of the lease, or (b) the date on which the Company commences occupancy of any portion of the premises for the permitted uses under the lease. The Company has options to extend the term of the lease for two additional successive periods of five years each (the "Extension Periods").

Annual fixed rent will be approximately $3.9 million for the first 12-month period during the Original Term, increasing on an annual basis until reaching approximately $4.7 million for the seventh 12-month period during the Original Term. The Lexington Lease provides for an aggregate fixed rent of approximately $30.1 million during the seven-year Original Term. Annual fixed rent during the Extension Periods will be agreed upon between the Company and the Landlord following the Company's provision of notice of its intention to exercise an extension option. If the Company and the Landlord cannot agree on annual fixed rent during an Extension Period, the Company will have the right to seek, subject to the terms of the Lexington Lease, a broker determination of the prevailing market rent, and the annual fixed rent during such Extension Period will be the prevailing market rent determined by the broker.

In addition to the annual fixed rent, the Company will be responsible for certain customary operating expenses and real estate taxes specified in the agreement. The Lexington Lease also contains customary default provisions allowing the landlord to terminate the lease or seek damages if the Company fails to cure certain breaches of its obligations under the lease within specified periods of time. In addition, the Company will be obligated to indemnify the landlord for certain losses incurred in connection with the Company's use or occupancy of the premises.

Cambridge sublease

On January 4, 2019, the Company entered into a non-cancelable real property sublease agreement with PPF OFF 150 Cambridge Park Drive, LLC (the "Landlord") and International Business Machines Corporation (the "Sublandlord"), for approximately 9,653 square feet of office space in Cambridge, Massachusetts ("Cambridge Sublease"). The term of the sublease commenced on January 11, 2019, the date that the Landlord provided written consent to the Cambridge Sublease, and extends through the sublease expiration date of July 30, 2021. The Cambridge Sublease provides for an aggregate fixed rent of approximately $0.8 million during the term of the sublease.

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

None.

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ITEM 9A. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our periodic and current reports that we file under the Exchange Act, with the SEC is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our chief executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

As of the end of the period covered by this Annual Report on Form 10-K, we carried out an evaluation, under the supervision and with the participation of our management, including the chief executive officer and the chief financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15. Based upon, and as of the date of, this evaluation, our chief executive officer and our chief financial officer concluded that our disclosure controls and procedures were effective. Accordingly, management believes that the financial statements included in this report fairly present in all material respects our financial condition, results of operations and cash flows for the periods presented.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act). Under the supervision and with the participation of our management, including our chief executive officer and chief financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2018 based on the guidelines established in Internal Control—Integrated Framework 2013 issued by the Committee of Sponsoring Organizations of the Treadway Commission. Our internal control over financial reporting includes policies and procedures that provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external reporting purposes in accordance with U.S. generally accepted accounting principles.

Based on that evaluation, management concluded that our internal control over financial reporting was effective as of December 31, 2018.

Attestation Report of the Registered Public Accounting Firm

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm as we are a smaller reporting company and an "emerging growth company" as of December 31, 2018, as defined in the Jumpstart Our Business Startups Act of 2012.

Changes in Internal Control Over Financial Reporting

We continuously seek to improve the efficiency and effectiveness of our internal controls. This results in refinements to processes throughout the Company. There was no change in our internal control over financial reporting during the quarter ended December 31, 2018, which was identified in connection with our management's evaluation required by Exchange Act Rules 13a-15(f) and 15d-15(f) that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on the Effectiveness of Controls

Our management, including the chief executive officer and chief financial officer, does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within a company have been detected. These inherent limitations include the realities that judgments in decision making can be faulty and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by management override of the control. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions. Over time, controls may become inadequate because of changes in conditions, or the degree of compliance with the policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

ITEM 9B. OTHER INFORMATION

None.

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PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this item and not set forth below will be set forth in the definitive proxy statement (the "Proxy Statement") for our 2019 Annual Meeting of Stockholders to be filed with the SEC pursuant to Regulation 14A not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, and is incorporated herein by reference.

Information regarding our audit committee financial expert will be set forth in the Proxy Statement and is incorporated herein by reference.

We have adopted a Code of Business Conduct and Ethics applicable to all employees, including the principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions. The Code of Business Conduct and Ethics is posted on our website at www.dicerna.com. Amendments to, and waivers from, the Code of Business Conduct and Ethics that apply to any of these officers, or persons performing similar functions, and that relate to any element of the code of ethics definition enumerated in Item 406(b) of Regulation S-K will be disclosed at the website address provided above and, to the extent required by applicable regulations, on a Current Report on Form 8-K.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this item will be set forth in the Proxy Statement and is incorporated herein by reference.

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

The information required by this item will be set forth in the Proxy Statement and is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

The information required by this item will be set forth in the Proxy Statement and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information required by this item will be set forth in the Proxy Statement and is incorporated herein by reference.

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PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(1) Consolidated Financial Statements:

The following consolidated financial statements are filed as part of this Annual Report on Form 10-K under Item 8 – "Financial Statements and Supplementary Data."

	Page
Report of Independent Registered Public Accounting Firm	83
Consolidated Balance Sheets	84
Consolidated Statements of Operations	85
Consolidated Statements of Changes in Redeemable Convertible Preferred Stock and Stockholders' Equity	86
Consolidated Statements of Cash Flows	87
Notes to Consolidated Financial Statements	89

(2) Financial Statement Schedules.

All schedules are omitted because they are not applicable, not required, or because the required information is included in the consolidated financial statements or notes thereto.

(3) Exhibits.

EXHIBIT INDEX

		Incorporated by Reference			
Exhibit Number	Description of Documents	Form	File Number	Exhibit	Filing Date
3.1	Amended and Restated Certificate of Incorporation of the Company.	8-K	001-36281	3.1	February 5, 2014
3.2	Amended and Restated Bylaws of the Company.	8-K	001-36281	3.2	February 5, 2014
3.3	Certificate of Designation of Redeemable Convertible Preferred Stock.	8-K	001-36281	3.1	March 30, 2017
3.4	Certificate of Elimination of the Redeemable Convertible Preferred Stock, dated as of December 29, 2017.	8-K	001-36281	3.1	December 29, 2017
4.1	Specimen Common Stock Certificate.	S-1	333-193150	4.1	January 28, 2014
4.1A	Form of Redeemable Convertible Preferred Stock Certificate.	8-K	001-36281	4.1	March 30, 2017
4.2	Form of Warrant to Purchase Common Stock.	S-1	333-193150	4.2	December 31, 2013
4.3	Form of Warrant to Purchase Preferred Stock.	S-1	333-193150	4.3	December 31, 2013
4.4	Form of Amended and Restated Registration Rights Agreement.	8-K	001-36281	10.2	March 30, 2017
4.4A	Form of First Amendment to Registration Rights Agreement.	8-K	001-36281	10.1	December 18, 2017
10.1+	2007 Employee, Director and Consultant Stock Plan, as amended (the "2007 Plan").	S-1	333-193150	10.1	December 31, 2013
10.2+	Form of Restricted Stock Agreement under the 2007 Plan.	S-1	333-193150	10.2	December 31, 2013
10.3+	Form of Incentive Stock Option Agreement under the 2007 Plan.	S-1	333-193150	10.3	December 31, 2013
10.4+	Form of Non-Qualified Stock Option Agreement under the 2007 Plan.	S-1	333-193150	10.4	December 31, 2013
10.5+		S-1	333-193150	10.5	

	2010 Employee, Director and Consultant Equity Incentive Plan, as amended (the "2010 Plan").				December 31, 2013
10.6+	Form of Stock Option Grant Notice and Stock Option Agreement under the 2010 Plan.	S-1	333-193150	10.6	December 31, 2013
10.7+	Form of Restricted Stock Agreement under the 2010 Plan.	S-1	333-193150	10.7	December 31, 2013
10.8+	2014 Employee Stock Purchase Plan.	S-1	333-193150	10.9	January 28, 2014
10.9+	Form of Indemnification Agreement by and between the Company and each of its directors.	S-1	333-193150	10.10	January 28, 2014
10.10+	Letter agreement dated as of June 2, 2009, by and between the Company and David M. Madden.	S-1	333-193150	10.14	December 31, 2013
10.11+	Letter agreement dated as of February 28, 2011, by and between the Company and Dennis H. Langer M.D., J.D.	S-1	333-193150	10.15	December 31, 2013
10.12	Lease agreement dated as of July 11, 2014, by and between the Company and King 87 CPD LLC.	10-Q	001-36281	10.5	November 6, 2014
10.13+	Letter Agreement dated as of September 12, 2014, by and between the Company and Bruce Peacock.	10-K	001-36281	10.26	March 12, 2015

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Exhibit Number	Description of Documents	Incorporated by Reference			
		Form	File Number	Exhibit	Filing Date
10.14	Sales Agreement, dated as of March 12, 2015, between the Registrant and Cowen and Company, LLC.	S-3	333-202687	1.2	March 12, 2015
10.15+	Amended and Restated 2014 Performance Incentive Plan.	8-K	001-36281	10.1	July 7, 2015
10.16+	Form of Incentive Stock Option Agreement under the Amended and Restated 2014 Performance Incentive Plan.	10-K	001-36281	10.31	March 10, 2016
10.17+	Form of Non-Qualified Stock Option Agreement under the Amended and Restated 2014 Performance Incentive Plan.	10-K	001-36281	10.32	March 10, 2016
10.18+	Separation Agreement dated as of December 15, 2015 by and between the Company and James E. Dentzer.	10-K	001-36281	10.33	March 10, 2016
10.19+	Offer Letter dated as of January 14, 2016 by and between the Company and John “Jack” Green.	10-K	001-36281	10.34	March 10, 2016
10.20+	Dicerna Pharmaceuticals, Inc. 2016 Inducement Plan.	S-8	333-210071	4.3	March 10, 2016
10.21+	Form of Dicerna Pharmaceuticals, Inc. Non-Qualified Inducement Stock Option Agreement.	S-8	333-210071	4.2	March 10, 2016
10.22+	Form of Non-Plan Inducement Stock Option Agreement.	S-8	333-210071	4.4	March 10, 2016
10.23+	Amended and Restated Employment Agreement dated as of July 8, 2016 by and between the Company and Douglas M. Fambrough, III.	10-Q	001-36281	10.1	November 7, 2016
10.24+	Amended and Restated Employment Agreement dated as of July 8, 2016 by and between the Company and Bob. D. Brown.	10-Q	001-36281	10.2	November 7, 2016
10.25+	Amended and Restated Employment Agreement dated as of July 6, 2016 by and between the Company and James B. Weissman.	10-Q	001-36281	10.3	November 7, 2016
10.26+	Amended and Restated Employment Agreement dated as of November 4, 2016 by and between the Company and John B. Green.	10-Q	001-36281	10.4	November 7, 2016
10.27	Form of Letter Agreement by and between the Company and Adam Koppel.	8-K	001-36281	10.3	March 30, 2017
10.28	Form of Redeemable Convertible Preferred Stock Purchase Agreement by and among the Company and seven institutional investors led by funds advised by Bain Capital Life Sciences L.P.	8-K	001-36281	10.1	March 30, 2017
10.29+	Employment Agreement, dated May 18, 2017, by and between the Company and Ralf Rosskamp.	10-Q	001-36281	10.3	August 10, 2017
10.30	Collaborative Research and License Agreement, dated October 27, 2017, by and between the Company and Boehringer Ingelheim International GmbH.	10-K	001-36281	10.30	March 8, 2018
10.31	Letter Agreement entered into on December 13, 2017 by and between the Company and the holders of its redeemable convertible preferred stock.	8-K	001-36281	10.1	December 14, 2017
10.32†	Confidential Settlement Agreement and General Release, dated April 18, 2018, between the Company and Alnylam Pharmaceuticals, Inc.	10-Q	001-36281	10.1	August 8, 2018
10.33		10-Q	001-36281	10.2	

	Share Issuance Agreement, dated April 20, 2018, between the Company and Alnylam Pharmaceuticals, Inc.	August 8, 2018
10.34†*	Collaborative Research and License Agreement, dated October 22, 2018, by and between the Company and Alexion Pharma Holding Unlimited Company.	
10.35*	Alexion Share Issuance Agreement, dated October 22, 2018, by and between the Company and Alexion Pharma Holding Unlimited Company.	
10.36†*	Collaboration and License Agreement, dated October 25, 2018, by and between the Company and Eli Lilly and Company.	
10.37*	Lilly Share Issuance Agreement, dated October 25, 2018, by and between the Company and Eli Lilly and Company.	
10.38†*	Additional Target Agreement, dated December 31, 2018, by and between the Company and Boehringer Ingelheim International GmbH.	
21.1*	Subsidiaries of the Company.	
23.1*	Consent of Independent Registered Accounting Firm.	
24	Power of Attorney (reference is made to the signature page).	
31.1*	Certification of the Company's principal executive officer required by Rule 13a-14(a) or Rule 15d-14(a).	
31.2*	Certification of the Company's principal financial officer required by Rule 13a-14(a) or Rule 15d-14(a).	
32.1**	Section 1350 Certifications.	
101.INS*	XBRL Report Instance Document	
101.SCH*	XBRL Taxonomy Extension Schema Document	
101.CAL*	XBRL Taxonomy Calculation Linkbase Document	
101.LAB*	XBRL Taxonomy Label Linkbase Document	
101.PRE*	XBRL Taxonomy Presentation Linkbase Document	
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document	

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† Confidential treatment with respect to specific portions of this Exhibit has been requested, and such portions are omitted and have been filed separately with the Securities and Exchange Commission.

+ Management contract or compensatory plan or arrangement.

* Filed herewith.

** Exhibit 32.1 is being furnished and shall not be deemed to be "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liability of that section, nor shall such exhibit be deemed to be incorporated by reference in any registration statement or other document filed under the Securities Act, or the Exchange Act, except as otherwise stated in such filing.

ITEM 16. FORM 10-K SUMMARY

None.

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SIGNATURES

Pursuant to 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, in Cambridge, Commonwealth of Massachusetts on March 13, 2019.

By: /s/ Douglas M. Fambrough, III
Douglas M. Fambrough, III, Ph.D.
Chief Executive Officer and Director
(Principal Executive Officer)

By: /s/ John B. Green
John B. Green
Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)

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POWER OF ATTORNEY

KNOW ALL PERSON BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Douglas M. Fambrough, III, Ph.D. and John B. Green and each of them, as his true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution, for him and in his name, place and stead, in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as he might or could do in person, hereby ratify and confirming all that said attorneys-in-fact and agents, or any of them, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this report has been signed by the following persons in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Douglas M. Fambrough, III Douglas M. Fambrough, III, Ph.D.	Chief Executive Officer and Director (Principal Executive Officer)	March 13, 2019
/s/ John B. Green John B. Green	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	March 13, 2019
/s/ Kevin Buchi Kevin Buchi	Chairman	March 13, 2019
/s/ Anna Protopapas Anna Protopapas	Director	March 13, 2019
/s/ Martin Freed Martin Freed, M.D.	Director	March 13, 2019
/s/ Brian K. Halak Brian K. Halak, Ph.D.	Director	March 13, 2019
/s/ Stephen J. Hoffman Stephen J. Hoffman, MD., Ph.D.	Director	March 13, 2019
/s/ Peter Kolchinsky Peter Kolchinsky, Ph.D.	Director	March 13, 2019
/s/ Adam M. Koppel Adam M. Koppel, M.D., Ph.D.	Director	March 13, 2019
/s/ Marc Kozin Marc Kozin	Director	March 13, 2019
/s/ Dennis H. Langer Dennis H. Langer, M.D., J.D.	Director	March 13, 2019
/s/ Cynthia Smith	Director	March 13, 2019

Cynthia Smith

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