ARQULE INC
Form 10-K
March 07, 2019
TABLE OF CONTENTS

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

FORM 10-K
ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF
THE SECURITIES EXCHANGE ACT OF 1934
FOR THE FISCAL YEAR ENDED DECEMBER 31, 2018 COMMISSION FILE NUMBER: 000-21429
ARQULE, INC.
(EXACT NAME OF REGISTRANT AS SPECIFIED IN ITS CHARTER)

DELAWARE	04-3221586
(STATE OR OTHER JURISDICTION OF INCORPORATION OR ORGANIZATION)	(I.R.S. EMPLOYER IDENTIFICATION NO.)

ONE WALL STREET, BURLINGTON, MASSACHUSETTS 01803
(ADDRESS OF PRINCIPAL EXECUTIVE OFFICES INCLUDING ZIP CODE)
REGISTRANT'S TELEPHONE NUMBER, INCLUDING AREA CODE:
(781) 994-0300
SECURITIES REGISTERED PURSUANT TO SECTION 12(b) OF THE ACT:

(TITLE OF EACH CLASS)	NAME OF EACH EXCHANGE ON WHICH REGISTERED
COMMON STOCK, $.01 PAR VALUE	The NASDAQ Stock Market LLC (NASDAQ Global Market)

SECURITIES REGISTERED PURSUANT TO SECTION 12(g) OF THE ACT:
NONE
Indicate by check mark if the registrant is a well-known issuer, as defined in Rule 405 of the Securities Act. Yes No
Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes No
Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No
Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405) of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No
Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See definitions of "large accelerated filer," "accelerated filer," and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

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

Indicate If an emerging growth company, indicate by check by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to

Section 13(a) of the Exchange Act.

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

The aggregate market value of voting and non-voting common stock held by non-affiliates of the registrant as of June 30, 2018 was: $531,446,974.

There were 109,003,637 shares of the registrant's common stock outstanding as of February 20, 2019.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the definitive proxy statement for the Registrant's Annual Meeting of Stockholders to be held on May 14, 2019 which will be filed with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year end of December 31, 2018, are incorporated by reference into Part III of the Form 10-K.

TABLE OF CONTENTS

FORWARD-LOOKING STATEMENTS

You should carefully consider the risks described below together with all of the other information included in this Form 10-K, including Item 1A “Risk Factors,” before making an investment decision. An investment in our common stock involves a high degree of risk. We operate in a dynamic and rapidly changing industry that involves numerous uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the trading price of our common stock to decline, and you may lose all or part of your investment.

This Form 10-K, including information incorporated herein by reference, contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995. All statements that are not descriptions of historical fact are forward-looking statements, based on estimates, assumptions and projections that are subject to risks and uncertainties. These statements can generally be identified by use of forward-looking terminology such as “believes”, “expects”, “intends”, “may”, “will”, “plans”, “should”, “anticipates,” “potential,” “goal” or similar terminology. Although we b the expectations reflected in such forward looking statements are reasonable as of the date of this Form 10-K, such expectations are based on certain assumptions regarding the progress of product development efforts including clinical trials and preclinical activities conducted by ourselves and third parties, the prosecution of existing and efforts to execute new collaborative agreements, receipt of potential milestones and royalties under our collaborative agreements, government regulations, reliance on third parties to conduct clinical trials and perform research and analysis services, adequate financial resources, changes in economic and business conditions, and other factors relating to our growth. Such expectations may not materialize if product development efforts, including any necessary trials of our potential product candidates, are delayed or suspended, if our product candidates fail to demonstrate safety and efficiency, if positive early results are not repeated in later studies or in humans, if the therapeutic value of our compounds is not realized, if planned acquisitions or negotiations with potential collaborators are delayed or unsuccessful, if we are unsuccessful at integrating acquired assets or technologies, or if other assumptions prove incorrect. The forward-looking statements contained herein represent the judgment of ArQule as of the date of this Form 10-K. ArQule disclaims any intent or obligation to update any forward-looking statement except to the extent required by law.

TABLE OF CONTENTS
ARQULE, INC.
TABLE OF CONTENTS

3

TABLE OF CONTENTS

PART I

ITEM 1. BUSINESS

OVERVIEW

We are a biopharmaceutical company engaged in the research and development of innovative therapeutics to treat cancers and rare diseases. Our mission is to discover, develop and commercialize novel small molecule drugs in areas of high unmet need that will dramatically extend and improve the lives of our patients. These product candidates target biological pathways implicated in a wide range of cancers and certain non-oncology indications. Our discovery and development efforts are guided, when possible, by an understanding of the role of biomarkers, which are indicators of a particular biological condition or process and may predict the clinical benefit of our compounds in defined patient populations. Our clinical-stage pipeline consists of four product candidates, all of which are in targeted patient populations, making ArQule a leader among companies our size in precision medicine.

ArQule has a long history of kinase drug discovery and development, having discovered and introduced ten kinase inhibitors into clinical trials. Our drug discovery efforts have been informed by our historical expertise in chemistry, our work in rational drug design and by our insight into kinase binding and regulation. We have applied this knowledge to produce significant chemical matter for a number of kinase targets and to build an extensive library of proprietary compounds with the potential to target multiple kinases in oncology and other therapeutic areas, such as rare diseases. We may bring further preclinical programs forward and interrogate our library against new targets beyond kinases either directly or with collaborators.

Our proprietary pipeline of orally bioavailable product candidates is directed toward molecular targets and biological processes with demonstrated roles in the development of both human cancers and rare, non-oncology diseases. All of these programs are being developed in targeted, biomarker-defined patient populations. By identifying subgroups of patients that are most likely to respond to our product candidates, we seek to target small, often orphan, indications that allow for focused and efficient development. In addition to pursuing these potentially fast-to-market strategies, we also pursue development in other indications that could allow us to expand the utility of the product candidates, if approved. Our clinical pipeline includes the following product candidates:

- ARQ 531 is a potent and reversible dual inhibitor of both wild type and C481S-mutant Bruton’s tyrosine kinase (BTK) that is in Phase 1 clinical development for B-cell malignancies refractory to other therapeutic options

- Miransertib (ARQ 092) is a potent and selective inhibitor of protein kinase B (AKT), a serine/ threonine kinase. We expect to commence a registrational clinical trial of miransertib for the treatment of Proteus syndrome and PIK3CA-Related Overgrowth Syndromes (PROS) in the first half of 2019. Miransertib is also in Phase 1b clinical development in oncology in combination with the hormonal therapy, anastrozole

- ARQ 751 is a next-generation, highly potent and selective inhibitor of AKT that is in Phase 1 clinical development for solid tumors harboring AKT, phosphoinositide 3-kinase (PI3K) or phosphatase and tensin homolog (PTEN) mutations or that are PTEN null

- Derazantinib (ARQ 087) is a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family of kinases that is in a registrational clinical trial in intrahepatic cholangiocarcinoma (iCCA) in patients with FGFR2 fusions. Derazantinib was exclusively licensed to Basilea Pharmaceutica Limited (Basilea) in April 2018 in the United States, European Union, Japan and the rest of the world, excluding the People’s Republic of China, Hong Kong, Macau, and Taiwan (collectively, Greater China) where derazantinib was exclusively licensed to Sinovant Sciences Ltd., a subsidiary of Roivant Sciences Ltd. (Sinovant) in February 2018

TABLE OF CONTENTS

OUR STRATEGY

Our strategy is to build a commercial-stage biotechnology company that uses precision medicine to develop small molecule drugs in biomarker-defined patient populations where such drugs are likely to have the greatest clinical benefit. Specifically, we intend to accomplish this through the following activities:

- Advance proprietary pipeline programs to achieve rapid proof of principle and regulatory approval. ARQ 531 is in a dose escalation clinical trial in patients with highly refractory B-cell malignancies where it will have the opportunity to show rapid proof of principle in the biomarker-defined C481S-mutant BTK patient population. We expect miransertib to be in a registrational clinical trial in the first half of 2019 in two rare, genetically defined overgrowth diseases. Derazantinib is in a potential fast-to-market registrational clinical trial in approximately 100 patients with iCCA with FGFR2 fusions in collaboration with Basilea and Sinovant.

- Pursue precision medicine. We pursue precision medicine approaches with our proprietary pipeline to define patient populations with the highest likelihood of benefitting from our therapies based on our insights into functional biomarkers, with the goal of achieving greater speed, efficiency and enhanced outcomes in the development process. All of our product candidates are being developed in clinical trials with biomarker-defined populations.

- Focus on cancer, a market with a large unmet need. Cancer is the second most common cause of death in the United States. According to the American Cancer Society, in 2019 approximately 607,000 cancer-related deaths are projected to occur and more than 1.7 million new cases of cancer are projected to be diagnosed in the United States. Demographic trends and improved screening are expected to increase the rate of cancer diagnoses, as approximately 77 percent of cancers occur in the over-55-year-old population.

- Expand our efforts in rare diseases. We have expanded beyond oncology into rare disease indications by utilizing our oncology expertise in targets that are common to both disease settings and by collaborating with the academic leaders in these fields. In all instances, we pursue a biomarker-defined precision medicine strategy in areas of high unmet need that also provides the opportunity for accelerated development. We expect to to launch a registrational clinical trial in Proteus syndrome and PROS in the first half of 2019.

- Continue to expand diagnostic expertise through collaborations. We have extensive experience partnering with diagnostic companies for clinical trials in many parts of the world. In early clinical testing, we often utilize existing diagnostic technologies to identify patient subsets with the highest likelihood of clinical benefit. In later-stage clinical development, particularly in registrational trials such as the derazantinib trial in iCCA with Basilea, we collaborate with experienced diagnostic partners to support our clinical and commercial precision medicine strategy.

- Benefit from the resources and strength of our collaborators. We pursue alliances for our programs with pharmaceutical and biotechnology companies, as well as research institutions and independent investigators, to finance operations, offset spending, balance risk and gain expertise.

TABLE OF CONTENTS

GOALS FOR PIPELINE DEVELOPMENT

The chart below summarizes the current stage of our pipeline of product candidates.

OUR PRODUCT CANDIDATES

BTK Program: ARQ 531

Overview

ARQ 531 is an investigational, orally bioavailable, potent and reversible dual inhibitor of both wild type and C481S-mutant BTK. BTK is a key component of the B-cell receptor (BCR) signaling pathway and has emerged as a critical target in the treatment of B-cell malignancies. The leading approved BTK inhibitor, ibrutinib, improves survival in chronic lymphocytic leukemia (CLL) compared to standard chemotherapy or immune therapy. However, in a subset of patients, a somatic mutation (C481S) of the covalent binding site results in acquired resistance to ibrutinib therapy and in poor clinical outcomes for these patients.

ARQ 531 has demonstrated promising activity in both in vitro and in vivo models. These data suggest that ARQ 531 could be effective against both wild type and C481S-mutant BTK, as well as other indications where ibrutinib is not highly effective. We intend to pursue an expedited development strategy in patients with the C481S mutation and explore other indications in B-cell malignancies where ARQ 531 shows the greatest promise. We presented data from the first six cohorts of a dose escalation clinical trial of ARQ 531 at the American Society of Hematology (ASH) conference in December 2018.

Background on BTK Inhibitors

B-cell malignancies, such as CLL, diffuse large B-cell lymphoma (DLBCL) and mantle cell lymphoma (MCL) are driven by BTK. The leading BTK inhibitor, ibrutinib, is irreversible and makes a covalent bond with the C481 residue of the targeted protein. Although ibrutinib has demonstrated excellent responses in patients with elevated BCR signaling, clinical resistance has been observed, and the BTK C481S-mutation that prevents covalent binding of ibrutinib to BTK is emerging as a predominant mechanism of resistance.

Currently it is estimated that approximately 20% of patients treated with ibrutinib become refractory, or resistant, to ibrutinib, and this incidence is expected to grow as more patients are prescribed ibrutinib and patient time on therapy increases. The BTK-C481S mutation is the most prevalent resistance mechanism for patients who become refractory to ibrutinib, representing approximately 85% of refractory cases in CLL. Currently there is no approved targeted therapy for ibrutinib refractory patients with the C481S mutation.

ARQ 531 is a highly optimized, small molecule, reversible inhibitor of C481S-mutant BTK and wild type BTK. As a reversible inhibitor, ARQ 531 does not require interaction with the C481 residue, a binding site essential for irreversible BTK inhibitors, thus potentially positioning ARQ 531 as a targeted therapy for CLL, DLBCL and MCL patients harboring C481S-mutant BTK who have developed resistance to

TABLE OF CONTENTS

irreversible BTK inhibitors. ARQ 531 has also demonstrated strong signs of preclinical activity in wild type BTK models, including the highly predictive TCL1 mouse model conducted by our collaborators at The Ohio State University. These and other data suggest additional development opportunities for ARQ 531.

Key Characteristics of ARQ 531

- Oral, reversible dual inhibitor of both wild type and C481S-mutant BTK
- Potently inhibits activation of the C481S-mutant BTK with long residence time
- Showed remarkable efficacy in in vivo TCL1 mouse model and a substantial survival benefit compared to ibrutinib
- Good absorption, distribution, metabolism, and excretion (ADME) profile and excellent oral bioavailability in several species
- Predictable pharmacokinetic (PK) profile in patients that supports once daily (QD) dosing
- Composition of matter patent protection through December 2035

Preclinical Development of ARQ 531

In August 2018, we and our collaborators at The Ohio State University published a comprehensive paper in Cancer Discovery detailing the findings from multiple in vitro and in vivo studies with ARQ 531. This paper also depicts the chemical structure of ARQ 531 and its crystal structure as resident in the binding pocket of BTK, which substantially explains its ability to inhibit both wild type and C481S-mutant BTK.

Specifically, the paper demonstrated that in vitro treatment of CLL cells with ARQ 531 decreases BTK-mediated functions including BCR signaling, viability and migration. In vivo, ARQ 531 was found to increase survival over ibrutinib in an engraftment mouse model of CLL and in an engraftment mouse model resembling Richter's transformation.

Clinical Development of ARQ 531

We filed an investigational new drug application (IND) for ARQ 531 in the first quarter of 2017 and commenced a Phase 1a/b clinical trial in the third quarter of 2017 to study the safety of ARQ 531, look for signs of activity in a number of indications, including in CLL patients harboring the BTK-C481S mutation, and to identify a therapeutic dose.

In December 2018, we presented interim safety and efficacy data from the initial six cohorts of the Phase 1a portion of the clinical trial at the ASH conference. The interim data demonstrated a manageable safety profile for ARQ 531 and anti-tumor activity was observed in nine out of 20 patients. Subject to the receipt of additional data, we expect to establish a recommended Phase 2 dose for ARQ 531 in 2019 and thereafter to enroll a number of expansion cohorts at the recommended Phase 2 dose. For later stage clinical testing, we initially plan to pursue a fast-to-market strategy in patients with the C481S mutation. In addition, because ARQ 531 potently inhibits wild type BTK and other kinases relevant to B-cell malignancies, we expect to evaluate ARQ 531 in other indications where ibrutinib is not highly effective and where ARQ 531 could be superior. We plan to report additional clinical data on ARQ 531 at a major medical conference in 2019.

AKT Program: Miransertib (ARQ 092) and ARQ 751

Overview

Miransertib (ARQ 092) and our next generation AKT inhibitor, ARQ 751, are oral, potent and selective inhibitors of the AKT serine/threonine kinase. AKT1, AKT2 and AKT3 are key signaling protein kinases of the PI3K/AKT/mTOR

pathway that are involved in processes associated with cancer such as cell proliferation, migration, survival and protein synthesis. Activation of this pathway is common in many cancers, suggesting that AKT kinases are compelling targets for the treatment of oncology indications.

TABLE OF CONTENTS

Dysregulation of AKT is also a driver of certain rare proliferative disorders. For example, the E17K mutation of AKT1 causes Proteus syndrome, an ultra-rare condition characterized by the aberrant overgrowth of multiple tissues of the body. Patients with Proteus syndrome experience changes in the shapes of certain body structures over time, including abnormal, often asymmetric, massive growth (overgrowth) of the skeleton, skin, adipose tissue and central nervous system out of proportion to the rest of the body. Similarly, PROS is a group of rare disorders that cause overgrowth of parts of the body due to gain-of-function mutations in the PIK3CA gene, including Klippel-Trenaunay syndrome, CLOVES syndrome, megalencephalies, infiltrating facial lipomatosis and many others. Miransertib and ARQ 751 have been shown preclinically and clinically to inhibit AKT and PI3K cell signaling and therefore may provide the potential for much-needed treatment options for patients with these diseases.

Background on AKT inhibitors

There are currently no approved AKT inhibitors for the treatment of cancers or rare overgrowth diseases, although there are a number of product candidates in various stages of clinical development in oncology. Miransertib and ARQ 751 are investigational, allosteric inhibitors of AKT1, AKT2 and AKT3. Both molecules are derived from a proprietary chemical class with distinct ADME and PK properties. Because ARQ 092 and ARQ 751 do not bind to AKT at the adenosine triphosphate (ATP) binding site, they are differentiated from ATP-competitive inhibitors of AKT, and we believe may provide greater selectivity and possibly fewer toxicities.

Miransertib (ARQ 092)

Key Characteristics of Miransertib

- Potent, selective, allosteric pan-AKT inhibitor
- Clinical effect observed in a number of oncology patients showing response evaluation criteria in solid tumors (RECIST) responses
- Inhibition of AKT signaling observed in patients at low doses, presenting attractive profile for treatment of certain rare diseases
- Manageable safety profile
- Good drug-like properties

Preclinical Development of Miransertib

Preclinical studies of miransertib showed high potency against AKT1, AKT2 and AKT3, with nanomolar inhibition of downstream proteins. Cancer cells that harbor the E17K-AKT1 mutation, the H1047R-PIK3CA mutation or that are PTEN-null are the most sensitive to miransertib. Tumor growth inhibition was observed in human tumor xenograft mouse models and PDX models of endometrial cancer and breast cancer harboring these mutations. In other preclinical studies, miransertib demonstrated combinability with standard of care in multiple cancer types, including in combination with trametinib for endometrial cancer, and in combination with paclitaxel or trastuzumab for breast cancer.

Clinical Development of Miransertib – Non-oncology (Rare Diseases)

In October 2018, we and our collaborators presented preliminary clinical data at the American Society of Human Genetics conference demonstrating the potential utility of miransertib in both Proteus syndrome and PROS. The data demonstrated that miransertib was well tolerated with a demonstrated manageable toxicity profile. Data from two patients treated as part of our named patient/compassionate use program showed, with respect to the patient with Proteus syndrome, an improvement in symptoms including improved mobility/bone changes, and with respect to the patient with PROS, clinical stabilization and radiological improvement of disease. Preliminary results from an

open-label, phase 1/2 clinical trial of miransertib in patients with PROS showed evidence of clinical activity, as demonstrated by improvements in disease related symptoms and objective radiologic and photographic measures. In February 2019, we and

TABLE OF CONTENTS

our collaborators announced additional pharmacodynamic, safety and clinical activity data for miransertib in patients with Proteus syndrome. The data demonstrated good target engagement, tolerability and reductions in lesion size and pain, especially in children. We expect to launch a registrational clinical trial in Proteus syndrome and PROS in the first half of 2019.

Orphan Designation

In November 2015 and March 2018, we received orphan drug designation from the United States Food and Drug Administration (FDA) and European Medicines Agency (EMA), respectively, in Proteus syndrome. The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States and there is no reasonable expectation that the cost of developing and making the drug for this type of disease or condition will be recovered from sales in the United States. The EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). In each case, the orphan drug designation entitles a party to financial incentives and certain marketing exclusivity for products that receive commercial approval.

Rare Pediatric Disease Designation

In the fourth quarter of 2017 we received rare pediatric disease designation from the FDA in connection with Proteus syndrome. The designation provides the opportunity for us to apply for a rare pediatric disease priority review voucher. The FDA awards priority review vouchers to sponsors of rare pediatric disease product applications that meet specified criteria. Under this program and subject to certain limitations, a sponsor like ArQule who may receive approval for a drug in a rare pediatric disease may qualify for a voucher that can be redeemed to receive priority review of a subsequent marketing application for a different product. If received, the pediatric voucher confers a valuable benefit to companies working in areas where the commercial opportunity is not as great as in other diseases.

Fast Track Designation

In September 2018, the FDA granted fast track designation to miransertib for the treatment of PROS. The FDA's fast track program aims to expedite the development and review of drugs which treat serious or life-threatening conditions and have demonstrated the potential to address unmet clinical needs.

Clinical Development of Miransertib – Oncology

We completed a Phase 1 clinical trial of miransertib in which it demonstrated a manageable safety profile and showed single agent activity, achieving partial responses in a number of patients whose tumors harbor AKT1 or PI3K mutations. A Phase 1b clinical trial evaluating miransertib in combination with the aromatase inhibitor, anastrozole, in patients with advanced endometrial cancer is currently ongoing.

ARQ 751

Key Characteristics of ARQ 751

- Highly potent and selective allosteric pan-AKT inhibitor
- Differentiated toxicology profile from miransertib suggests improved therapeutic index
- Differentiated PK profile from miransertib may lead to more favorable dosing characteristics
- Prolonged growth inhibition in several tumor xenograft mouse models
- Good drug-like properties

9

TABLE OF CONTENTS

Preclinical Development of ARQ 751

ARQ 751 is our next generation AKT inhibitor. The profile of ARQ 751 is similar to miransertib but shows a 5 to 15 fold increase in potency across binding, biochemical and cellular assays. ARQ 751 also is differentiated at the ADME and PK levels, as it has lower volume of distribution and does not accumulate in tissues. In GLP-toxicity studies in rats and monkeys, ARQ 751 had fewer and less severe rashes compared to miransertib, a toxicity common to AKT inhibitors in development.

Clinical Development of ARQ 751

ARQ 751 is currently being tested in a Phase 1 clinical trial for solid tumors harboring AKT, PI3K, or PTEN mutations or that are PTEN null. In November 2018, we presented preliminary data from the dose escalation portion of this trial at the EORTC/AACR/NCI Symposium. The data showed that ARQ 751 had manageable toxicity at the recommended Phase 2 dose of 75 mg QD. ARQ 751 demonstrated preliminary anti-tumor activity in two patients who each achieved a partial response after eight weeks on treatment. In addition, eleven patients had stable disease. Based upon these preliminary results, we plan to continue clinical development of ARQ 751 as a monotherapy and/or in combination with other anti-cancer agents.

FGFR Program: Derazantinib (ARQ 087)

Overview

Derazantinib is an oral, multi-kinase inhibitor designed to preferentially inhibit the FGFR family of kinases with demonstrated activity in FGFR2 genetic alterations, including fusions. Fibroblast growth factors and their receptors tightly regulate key cellular behaviors, such as proliferation, cell differentiation, cell migration, cell survival and angiogenesis. FGFR dysregulation has been identified as a driver in a number of cancers, including iCCA, cholangiocarcinoma, bladder, endometrial, breast, gastric, lung and ovarian.

Given the high unmet need and activity observed in our Phase 1/2 clinical trial of derazantinib, we selected iCCA as our first indication in a registrational clinical trial. iCCA is a rare type of bile duct cancer that originates from the intrahepatic biliary ductal system and forms an intrahepatic mass. The disease is often diagnosed late because it presents as asymptomatic. There are currently no approved therapies for iCCA. Both the FDA and EMA have granted derazantinib orphan drug designation for iCCA. We have exclusively licensed derazantinib to Basilea in the United States, European Union, Japan and the rest of the world, excluding Greater China where derazantinib has been exclusively licensed to Sinovant.

Key Characteristics of Derazantinib

- Multi-kinase inhibitor that potently inhibits FGFR1, FGFR2 and FGFR3 with demonstrated clinical activity
- Positive response rate observed in biomarker-defined iCCA population with FGFR2 fusions
- Safety profile differentiated from other FGFR inhibitors
- Consistent drug exposure with once-a-day dosing regimen
- Drug profile allows for combinability

Clinical Development of Derazantinib

We previously conducted a Phase 1a and Phase 2 clinical trial of derazantinib for the treatment of patients with iCCA. We observed six partial responses out of 29 evaluable patients representing a 21% response rate. Additionally, an 83% disease control rate and median time on treatment of over 26 weeks were observed in the trials, and the drug demonstrated a manageable side effect profile.

We initiated a registrational, biomarker-driven trial in second-line iCCA patients with FGFR2 fusions in the fourth quarter of 2017 and transferred the trial to Basilea in connection with the exclusive license we granted to them in April 2018. The trial is designed to be single-arm, response rate driven and will enroll approximately 100 patients in the United States and European Union. In January 2019, Basilea announced

TABLE OF CONTENTS

the results of an interim analysis in the clinical trial. The interim analysis was conducted after 42 patients had been enrolled, with a subset of 29 evaluable patients who had at least one post-baseline imaging assessment. The objective response rate in the 29 evaluable patients was 21%. The disease control rate was 83%. The safety data obtained from all 42 patients was consistent with the results from previous clinical trials of derazantinib.

CORPORATE PARTNERSHIPS

Basilea Pharmaceutica Ltd.

In April 2018, we entered into a License Agreement with Basilea pursuant to which ArQule granted Basilea an exclusive license to develop and commercialize derazantinib in the United States, European Union, Japan and the rest of the world, excluding Greater China. Under the terms of the agreement, we received an upfront payment of $10 million and are eligible for up to $326 million in regulatory and commercial milestones. Upon commercialization, we are entitled to receive staggered royalties on future net sales of derazantinib ranging from the high-single digits to the mid-teens on direct sales and mid-single digits to low-double digits on indirect sales. Basilea will be responsible for all costs and expenses of development, manufacture and commercialization in its territory. Under certain circumstances, we may have the opportunity to promote derazantinib in the United States directly.

Sinovant Sciences Ltd.

In February 2018, we entered into a License Agreement with Sinovant pursuant to which ArQule granted Sinovant an exclusive license to develop and commercialize derazantinib in Greater China. The agreement provides for an upfront payment to ArQule of $3 million and a $2.5 million development milestone that was paid in the first quarter of 2019. We are also eligible for up to an additional $82 million in regulatory and sales milestones. Upon commercialization, we are entitled to receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in Greater China. Sinovant will be responsible for all costs and expenses of development, manufacture and commercialization in Greater China.

PATENTS AND PROPRIETARY RIGHTS

We rely principally on patent and trade secret protection for our intellectual property, both in the United States and other countries. While many patent applications have been filed in the United States, the European Union and other foreign countries with respect to our product candidates, many of these have not yet been issued or allowed. The patent positions of companies in the biotechnology industry and the pharmaceutical industry are highly uncertain and involve complex legal and factual questions. Therefore, we cannot predict the breadth of claims, if any, that may be allowed under any of our patent applications, or the enforceability of any of our issued patents.

Patents extend for varying periods according to the date of patent filing or grant and the legal term of patents in the various countries where patent protection is obtained. The actual protection afforded by a patent, which can vary from country to country, depends on the type of patent, the scope of its coverage and the availability of legal remedies in the country.

As and when needed to support our current or future research and development programs, we may from time to time obtain rights under patents and other intellectual property owned by other parties through permanent or limited duration licenses or assignments of relevant intellectual property. These may include exclusive and nonexclusive licenses from medical and academic institutions and industry sources as well as generally available commercial licenses. For our current clinical and research programs, we are not a party to any material intellectual property agreement under which we could lose access to a technology necessary to continue research and development of our products if we failed to fulfill our obligations thereunder. We anticipate that we will continue to seek intellectual property rights from external sources where the applicable technology complements our research and development efforts.

For our BTK program, we have issued patents and pending patent applications in the U.S. and foreign jurisdictions. We have one issued patent in the U.S. covering the composition of matter of ARQ 531 and pharmaceutical composition comprising ARQ 531. The expiration date of this patent is December 23, 2035.

TABLE OF CONTENTS

We have also filed patent applications in the U.S. and foreign jurisdictions covering analogs of ARQ 531. If these applications are issued as patents, they will expire in August 2037. In addition, we have a pending U.S. application covering solid forms of ARQ 531. Patents that result from this application will expire in August 2039.

With respect to our AKT program, we have issued patents and pending patent applications in the United States, the European Union and other foreign jurisdictions. For miransertib (ARQ 092), we have five issued patents in the United States covering the composition of matter of ARQ 092 and pharmaceutical composition comprising ARQ 092, polymorphs of ARQ 092 and its mesylate salt, processes of manufacturing ARQ 092, and the composition of matter of analogs of ARQ 092. These patents will expire between December 2031 and April 2035. We also have granted patents in the European Union, Japan and other foreign jurisdictions covering ARQ 092. These patents will expire between December 2030 and April 2035. For ARQ 751, we have two issued patents in the United States covering the composition of matter of ARQ 751 and analogs of ARQ 751 and pharmaceutical composition comprising ARQ 751 or analogs of ARQ 751. These patents will expire in June 2032. We also have granted patents in the European Union, Japan and other foreign jurisdictions covering ARQ 751. These patents will expire in June 2032. In addition, we have one issued patent in the United States covering methods of using ARQ 092 or ARQ 751, which will expire in September 2035. Moreover, we have a pending U.S. application covering pharmaceutical combinations comprising ARQ 092 or ARQ 751. Patents that result from this application will expire in November 2039.

With respect to derazantinib (ARQ 087), which we have partnered with Basilea and Sinovant, we have four issued patents in the United States covering the composition of matter of ARQ 087 and pharmaceutical composition comprising ARQ 087, solid forms of ARQ 087, and processes of manufacturing ARQ 087. These patents will expire between January 2031 and December 2036. We also have granted patents in the European Union, Japan and other foreign jurisdictions covering ARQ 087. These patents will expire in December 2029. Furthermore, our discovery of small molecule kinase inhibitors has led us to file numerous composition of matter patent applications in various countries.

COMPETITION

The pharmaceutical and biotechnology industries are highly competitive and characterized by rapid and continuous technological innovation. We face intense competition from organizations such as large pharmaceutical companies, companies and academic and research organizations. The major pharmaceutical and biotechnology organizations competing with us have greater capital resources, larger overall research and development staff and facilities and considerably more experience in drug development and commercialization. Consequently, we face competition on several fronts, including:

- for collaborators and investors;
- for recruitment and retention of highly qualified scientific and management personnel;
- for qualified subjects for our clinical studies of our product candidates, which may result in longer and more costly clinical trials;
- with competitors' drugs that may result in effective, commercially successful treatments for the same cancers we target; and
- for partners to co-develop and advance our product candidates through all stages of development.

In the area of small molecule anti-cancer therapeutics, we have identified a number of companies that have clinical development programs and focused research and development in small molecule approaches to cancer, including: AbbVie Inc., Amgen, Inc., Astellas Pharma, Inc., Array BioPharma Inc., AstraZeneca PLC, Celgene Corporation,

Curis, Inc., Exelixis, Inc., Eli Lilly and Company, Gilead Sciences, Inc., GlaxoSmithKline plc, Incyte Corporation, Infinity Pharmaceuticals, Inc., Johnson and Johnson, Merck, Merck KGaA, Novartis AG, Pfizer, Inc., Principia Biopharma, Inc., the Roche Group, Sunesis Pharmaceuticals, Inc., Takeda Pharmaceuticals Co. Ltd., and many others. With respect to ARQ 531, we are aware of a number of companies that are or may be pursuing different approaches to C481S-mutant BTK inhibition, including Aptose Biosciences Inc., Roche and Sunesis Pharmaceuticals. Moreover, numerous companies are also pursuing inhibitors of wild-type BTK,

TABLE OF CONTENTS

including AbbVie with its drug, IMBRUVICA, and AstraZeneca with its drug, CALQUENCE. Other companies with BTK inhibitors currently in development include Astra Zeneca, BeiGene Co., Ltd., Merck KGaA, Eli Lilly, Gilead, GlaxoSmithKline, Principia Biopharma and others. Other approved drugs that may compete to treat ibrutinib refractory patients, including patients with C481S-mutant BTK, include AbbVie's Bcl-2 inhibitor, VENCLEXTA, and Verastem Oncology's COPIKTRA.

With respect to miransertib (ARQ 092) in rare diseases, we believe Roche is developing taselisib for PIK3CA-Related Overgrowth Syndromes (PROS). Regarding miransertib in oncology, we are aware of a number of companies that are or may be pursuing different approaches to AKT inhibition, including Astra Zeneca, Bayer, Eli Lilly, Merck, Novartis, Rexahn Pharmaceuticals, Inc. and Roche. Moreover, numerous companies are pursuing inhibitors of PI3K and mTOR, two kinases in the PI3K-AKT-mTOR pathway. These drugs include Idelalisib, an approved PI3K inhibitor, and Everolimus, Temsirolimus and Rapamycin, each of which are approved mTOR inhibitors.

With respect to derazantinib (ARQ 087), we are aware of a number of companies that are or may be pursuing FGFR inhibition, including Astra Zeneca, Bayer, BioClin Therapeutics, Debiopharm Group, Boehringer Ingelheim International GmbH, Eisai Co., Ltd., Five Prime Therapeutics, Incyte, Johnson & Johnson, Novartis, Pfizer, Principia Biopharma, Servier and Taiho Oncology. With respect to iCCA, our lead indication for derazantinib, we are aware of a number of companies with products under development, including Agios Pharmaceuticals, Inc., Bayer Healthcare Pharmaceutical, Bristol-Myers Squibb, Cellact Pharma Gmbh, Concordia Healthcare, Dainippon Sumitomo Pharma Co., Ltd., Delcath Systems, Inc., Exelixis, InCyte, Novartis, Oncotherapy Services, Inc. and Spectrum Pharmaceuticals, Inc.

There can be no assurance that our competitors will not develop more effective or more affordable products or technology or achieve earlier product development and commercialization than ArQule, thus rendering our technologies and/or products obsolete, uncompetitive or uneconomical.

GOVERNMENT REGULATION

Virtually all pharmaceutical and biotechnology products that we or our collaborators develop will require regulatory approval by governmental agencies prior to commercialization. The nature and the extent to which these regulations apply vary depending on the nature of the products. In particular, human pharmaceutical products are subject to rigorous preclinical and clinical testing and other approval procedures by the FDA or the applicable regulatory authorities in countries other than the United States. Various federal and, in some cases, state statutes and regulations also govern or influence the manufacturing, safety, labeling, storage, record keeping and marketing of these products. The process of obtaining these approvals and the subsequent compliance with appropriate statutes and regulations are time consuming and require substantial resources, and the outcome of these regulatory activities is uncertain.

United States Government Regulation

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

- completion of preclinical laboratory tests, animal studies and formulation studies conducted according to Good Laboratory Practices (GLP) and other applicable regulations;

- submission to the FDA of an IND application, which must become effective before clinical trials in the United States may begin;

- performance of adequate and well-controlled clinical trials according to Good Clinical Practices (GCP) to establish the safety and efficacy of the proposed product candidate for its intended use;

- submission to the FDA of a new drug application (NDA);

-

satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product will be produced to assess compliance with current Good Manufacturing Practices (GMP); and

•
FDA review and approval of the NDA.

TABLE OF CONTENTS

Preclinical and Clinical Studies

Once a product candidate is identified for development, it enters the preclinical testing stage. Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as in vitro and animal studies to assess the potential for adverse events and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations for safety/toxicology studies. An IND sponsor must submit the results of the preclinical studies, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational product to humans and must become effective before human clinical trials may begin. Preclinical or nonclinical testing typically continues even after the IND is submitted.

An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time, the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Upon effectiveness of the IND, the product candidate can then be studied in human clinical trials to determine if the drug candidate is safe and effective. These clinical trials involve three separate phases that often overlap, can take many years and are expensive. These three phases, which are subject to considerable regulation, are as follows:

- Phase 1. The product candidate initially is introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and elimination. In the case of some drug candidates for severe or life-threatening diseases, such as cancer, especially when the drug 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 initiated in a limited patient population intended to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the drug candidate for specific targeted diseases and to determine dosage tolerance and optimal dosage; and

- Phase 3. Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk-benefit ratio of the drug candidate and provide an adequate basis for regulatory approval and product labeling.

Phase 1, Phase 2 and Phase 3 testing may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend a clinical trial at any time for a variety of reasons, including a finding that the healthy volunteers or patients are being exposed to an unacceptable health risk. All clinical trials must be conducted under the supervision of one or more qualified investigators in accordance with GCP. Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently in other situations, including the occurrence of serious adverse events. Information about certain clinical trials must be submitted within specific time-frames to the National Institutes of Health for public dissemination on the www.clinicaltrials.gov website.

FDA Review Process

The results of drug development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug candidate, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product candidate. The FDA reviews each NDA submitted to ensure that it is sufficiently complete for substantive review before it accepts it for filing. It may request additional information rather than accept an NDA 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 candidate is safe and effective for its intended use and whether its manufacturing is GMP-compliant to assure and preserve the drug candidate’s identity,

14

TABLE OF CONTENTS

strength, quality and purity. The FDA may refer the NDA to an advisory committee for review and recommendation as to whether the NDA 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. Before approving an NDA, the FDA will typically inspect the facility or facilities where the product candidate is manufactured and tested. Additionally, before approving an NDA, the FDA may inspect one or more clinical trial sites to assure compliance with GCP requirements. The FDA may require, as a condition of approval, restricted distribution and use, enhanced labeling, special packaging or labeling, expedited reporting of certain adverse events, pre-approval of promotional materials, restrictions on direct-to-consumer advertising or commitments to conduct additional research post-approval. The FDA will issue a complete response letter if the agency decides not to approve the NDA in its present form. A complete response letter usually describes all of the specific deficiencies in the NDA identified by the FDA. The complete response letter may require additional clinical data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a complete response letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application.

Expedited Review and Approval

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. The receipt of any of the following designations does not change the standards for approval but may expedite the development or approval process.

The FDA may grant “fast track” designation to products that treat serious diseases or conditions and demonstrate the potential to address an unmet medical need. Fast track is a process designed to facilitate the development and expedite the review of such products by providing, among other things, more frequent meetings with the FDA to discuss the product’s development plan and, in certain cases, rolling review of an NDA, which allows submission of individually completed sections of an NDA for FDA review before the entire submission is completed. Fast track designation does not ensure that a product will be developed more quickly or receive FDA approval.

The FDA may grant “accelerated approval” to products that have been studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit to patients over existing treatments. For accelerated approval, the product must have an effect on a surrogate endpoint or an intermediate clinical endpoint that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on irreversible morbidity and mortality. When approval is based on surrogate endpoints or clinical endpoints other than survival or morbidity, the sponsor is typically required to conduct additional post-approval clinical studies to verify and describe the clinical benefit. These studies are known as “confirmatory trials.” Approval of a drug may be withdrawn or the labeled indication of the drug changed if these trials fail to verify clinical benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with the drug.

The “breakthrough therapy” designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint. For drugs and biologics that have been designated as breakthrough therapies, robust FDA-sponsor interaction and communication can help to identify the most efficient and expeditious path for clinical development, which may help minimize the number of patients placed in ineffective control regimens.

The FDA may grant “priority review” status to products that, if approved, would provide significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions. Priority review is intended to reduce the time it takes for the FDA to review an NDA, with the goal to take action on the application within six months, compared to ten months for a standard review.

Post-Marketing Requirements

Following approval of a new product, the manufacturer and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and record-keeping activities, reporting of adverse experiences, complying with GMP, complying with promotion and advertising

TABLE OF CONTENTS

requirements, which include restrictions on promoting drugs for unapproved uses or patient populations (known as "off-label use") and limitations on industry-sponsored scientific and educational activities. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such uses. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Further, if there are any modifications to the drug, including changes in indications, labeling or manufacturing processes or facilities, the applicant may be required to submit and obtain FDA approval of a new NDA or NDA supplement, which may require the development of additional data or preclinical studies and clinical trials.

FDA regulations require that products be manufactured in specific approved facilities and in accordance with GMP. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with GMP. These manufacturers must comply with GMP regulations that require, among other things, quality control and quality assurance, the maintenance of records and documentation and the obligation to investigate and correct any deviations from GMP. Manufacturers and other entities involved in the manufacture and distribution of approved drugs or biologics are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMP and other laws. The discovery of violations, including failure to conform to GMP, could result in enforcement actions, and the discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved NDA, including recall of the affected product.

Orphan Drug Designation

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

If a product candidate that has orphan drug designation subsequently receives the first FDA approval for that drug for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication for seven years following marketing approval, except in certain very limited circumstances, such as if the later product is shown to be clinically superior to the protected orphan product through a demonstration of either greater effectiveness, greater safety or a major contribution to patient care. Orphan drug exclusivity, however, also could block the approval of our drug candidates for seven years if a competitor first obtains approval of an orphan-designated drug that is considered the "same drug" (as defined by the FDA) for the same orphan indication.

Patent Term Restoration

Upon approval, products may be entitled to certain kinds of exclusivity under applicable intellectual property and regulatory regimes. The Drug Price Competition and Patent Term Restoration Act of 1984 (commonly known as 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. The length of the patent extension is roughly based on 50 percent of the period of time from the filing of an IND for a compound to the submission of the NDA for such compound, plus 100 percent of the time period from NDA submission to regulatory approval. The extension, however, cannot exceed five years and the patent term remaining after regulatory approval cannot exceed 14 years.

If the FDA approves, pursuant to an NDA, a drug product that contains an active moiety not previously approved in an NDA (an NCE), the new product is typically entitled to five years of non-patent regulatory exclusivity. This exclusivity blocks the FDA from accepting for review an abbreviated new drug application (ANDA) or a 505(b)(2) NDA for a drug with the same active moiety, except that an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. Approval of an NDA or NDA supplement (sNDA) for a drug that does not qualify for NCE exclusivity may qualify for a 3-year period of "changes" exclusivity, provided approval of the NDA or sNDA is based on one or more new clinical studies conducted, or sponsored by the applicant, that the FDA considers

TABLE OF CONTENTS

essential to approval of the NDA or sNDA. This 3-year exclusivity only blocks the approval of ANDAs and 505(b)(2) NDAs for drugs with the same active moiety for the protected change for which the clinical study or studies were essential. If, in response to a written request from the FDA, an NDA applicant conducts one or more clinical studies of a drug in pediatric populations, the FDA may grant pediatric exclusivity, which extends by 180 days a drug's unexpired regulatory exclusivities and listed patents which, on the date the FDA accepts the sponsor's completed study reports, have at least nine months left until expiration.

Rare Pediatric Disease Priority Review Voucher Program

Under the Rare Pediatric Disease Priority Review Voucher program, the FDA may award a priority review voucher to the sponsor of an approved NDA for a product that treats or prevents a rare pediatric disease. The voucher entitles the sponsor to priority review of one subsequent marketing application. A voucher may be awarded only for an approved rare pediatric disease product application. A rare pediatric disease product application is an NDA for a product that treats or prevents a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. In general, the disease must affect fewer than 200,000 such individuals in the U.S. In addition, certain other conditions must be met, including the following: the NDA must be deemed eligible for priority review, the NDA must not seek approval for a different adult indication (i.e., for a different disease/ condition), the product must not contain an active ingredient that has been previously approved by the FDA, and the NDA must rely on clinical data derived from studies examining a pediatric population such that the approved product can be adequately labeled for the pediatric population. Before NDA approval, the FDA may designate a product in development as a product for a rare pediatric disease, but such designation is not required to receive a voucher.

To receive a rare pediatric disease priority review voucher, a sponsor must notify the FDA, upon submission of the NDA, of its intent to request a voucher. If the FDA determines that the NDA is a rare pediatric disease product application, and if the NDA is approved, the FDA will award the sponsor of the NDA a voucher upon approval of the NDA. The FDA may revoke a rare pediatric disease priority review voucher if the product for which it was awarded is not marketed in the U.S. within 365 days of the product's approval. The voucher, which is transferable to another sponsor, may be submitted with a subsequent NDA and entitles the holder to priority review of the accompanying NDA. The sponsor submitting the priority review voucher must notify the FDA of its intent to submit the voucher with the NDA at least 90 days prior to submission of the NDA and must pay a priority review user fee in addition to any other required user fee.

Companion Diagnostic Development and Approval

For some clinical candidates for which there is a valid predictive biomarker, a diagnostic test known as a companion diagnostic may need to be developed and cleared or approved in parallel with the drug in order to identify patients who are likely to respond favorably to the drug or identify patients likely to be at increased risk for serious adverse reactions as a result of treatment with the drug. In the United States companion diagnostics are regulated as medical devices, and as such, require either clearance or approval prior to commercialization. The level of risk combined with available controls to mitigate risk determines whether a companion diagnostic device requires Premarket Approval Application (PMA) approval or is cleared through the 510(k) premarket notification process. We expect the diagnostic tests being developed for our product candidates will likely be subject to the PMA approval process.

Other Healthcare Laws

In developing and commercializing our drug product candidates, we may also be subject to various other federal and state laws, including fraud and abuse laws and privacy laws. Laws to which we could be subject include, but are not limited to the following:

•

Anti-Kickback Statute: prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, or receiving remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchasing, ordering, leasing, arranging for, or recommending the purchasing, ordering, or leasing of, any good or service for which payment may be made, in whole or in part, under a federal health care program such as Medicare or Medicaid;

17

TABLE OF CONTENTS

- False Claims Act: the federal civil and criminal false claims laws, including the civil False Claims Act, and Civil Monetary Penalties Law, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, false or fraudulent claims for payment or knowingly making, using or causing to made or used a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government;

- HIPAA: HIPAA created additional federal criminal statutes that prohibit, among other things, executing a scheme to defraud any health care benefit program or making false statements relating to health care matters, and apply regardless of the payor (e.g., public or private);

- HIPAA and HITECH: HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which impose obligations on HIPAA covered entities and their business associates, including mandatory contractual terms and required implementation of administrative, physical and technical safeguards to maintain the privacy and security of individually identifiable health information

- Transparency Requirements: the federal physician transparency requirements known as the Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or the Affordable Care Act (ACA), and similar states laws, which requires manufacturers of drugs, medical devices, biological and medical supplies covered by Medicare, Medicaid, or State Children's Health Insurance Program (CHIP) to report annually to the Centers for Medicare & Medicaid Services information related to payments and other transfers of value made by that entity to physicians and other healthcare providers and their immediate family members and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and

- Analogous State, Local and Foreign Laws: analogous state, local and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader than similar federal laws, can apply to claims involving health care items or services regardless of payor, including commercial insurers, and are enforced by many different federal and state agencies as well as through private actions.

Foreign Regulation

We conduct clinical trials and market our products in numerous jurisdictions outside the United States. Most of these jurisdictions have clinical trial, product approval and post-approval regulatory processes that are similar in principle to those in the United States. Thus, whether or not we obtain FDA approval for a drug candidate, we must obtain approval by the comparable regulatory authorities of foreign countries or economic areas, such as the European Union, before we can commence clinical trials or 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.

European Union Review and Approval

Under European Union regulatory systems, a company may submit marketing authorization applications (MAA) under a centralized, decentralized or national procedure. The centralized procedure is compulsory for certain medicines including those produced by biotechnology processes, or those medicines intended to treat certain diseases such as AIDS, cancer, neurodegenerative disorders, or diabetes, or for orphan medicinal products, and is optional for those medicines that contain a new active substance, or where the product is highly innovative. The centralized procedure provides for the grant of a single MAA that is valid for all European Union member states. This MAA is

issued by the European Commission based on the opinion of the Committee for Medicinal Products for Human Use of the European Medicines Agency (EMA).

In addition to the centralized procedure, the European Union has a nationalized procedure, which requires a separate application to and approval determination by each country; a decentralized procedure, whereby applicants submit identical applications to several countries and receive simultaneous assessment

TABLE OF CONTENTS

and approval in those countries involved in the procedure; and a mutual recognition procedure, where applicants that already have an authorization for the E.U. market can submit further applications for additional countries to recognize that initial authorization.

Under the above described procedures, before granting the MAA, the EMA or the competent authorities of the European Union member states make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

European Union Exclusivity

In the European Union, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon grant of an MAA, and an additional two years of market exclusivity. The data exclusivity, if applicable, prevents regulatory authorities in the European Union from referencing a company's data in order to assess a generic application for eight years. After this time, generic MAAs can be submitted, however, the additional two years of market exclusivity means that the product cannot be placed on the market until the expiry of such additional two-year period.

The European Commission, assisted by the EMA's Committee for Orphan Medicinal Products grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following medicinal product approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

EMPLOYEES

As of December 31, 2018, we employed 36 people in Burlington, Massachusetts. Of that total, 21 are engaged in research and development and 15 in general and administration, and 9 hold PhDs, 4 hold MDs and 4 hold Masters Degrees in the sciences.

CERTAIN OTHER INFORMATION

We file annual, quarterly and current reports, proxy statements and other information with the Securities and Exchange Commission ("SEC"). The SEC maintains a website at http://www.sec.gov that contains reports, proxy and information statements and other information concerning filers. We also maintain a web site at http://www.arqule.com that provides additional information about our company and links to documents we file with the SEC. The contents of our website are not incorporated into this report. Our Corporate Governance Principles; the charters of the Audit Committee, the Compensation, Nominating and Governance Committee, and the Science Committee; and the Code of Conduct are also available on our website.

TABLE OF CONTENTS

EXECUTIVE OFFICERS

Set forth below is certain information regarding our current executive officers, including their respective ages as of February 1, 2019.

NAME	AGE	POSITION
Paolo Pucci	57	Chief Executive Officer and a Director
Peter S. Lawrence	55	President and Chief Operating Officer
Robert J. Weiskopf	68	Chief Financial Officer and Treasurer
Dr. Brian Schwartz	57	Chief Medical Officer

Paolo Pucci

Chief Executive Officer

Mr. Pucci joined ArQule as Chief Executive Officer and a member of the Board in June 2008 from Bayer A.G., where he served as Senior Vice President and President in charge of the Bayer-Schering Pharmaceuticals Global Oncology/Specialized Therapeutics Business Units. Previously, Mr. Pucci was senior vice president of Bayer Pharmaceuticals Global Specialty Business Unit, President of U.S. Pharmaceutical Operations and a member of the Bayer Pharmaceuticals Global Management Committee. At Bayer, Mr. Pucci was involved in a broad range of activities related to Nexavar® (sorafenib), an oral multiple kinase inhibitor used to treat liver and kidney cancers. These activities included clinical development, regulatory review, corporate alliance management, product launch and marketing. Mr. Pucci joined Bayer as head of its Italian Pharmaceutical operations in 2001. Prior to Bayer, Mr. Pucci held positions of increasing responsibility with Eli Lilly, culminating with his appointment as Managing Director, Eli Lilly Sweden AB. At Lilly, his responsibilities included operations, sales, marketing and strategic planning. In November 2011, Mr. Pucci was appointed to the Board of Directors of Dyax Corp where he served as an independent director, member of the audit committee and chairman of the governance and nomination committee until the acquisition of Dyax by Shire in January 2016. In April 2013, he was appointed to the Board of Directors of Algeta ASA, an oncology company based in Oslo, Norway, where he served as an independent director and member of the audit committee until the acquisition of Algeta by Bayer A.G. He has also been a Director of NewLinks Genetics Corp., since November 2015. During September 2016, Mr. Pucci was elected to the Board of Directors of West Pharmaceutical Services, Inc., an international manufacturer of packing components and delivery systems for injectable drugs and healthcare products. Mr. Pucci holds an M.B.A from the University of Chicago, and is a graduate of the Università Degli Studi Di Napoli in Naples, Italy. He is also a chartered “Dottore Commercialista” in Italy.

Peter S. Lawrence

President and Chief Operating Officer

Mr. Lawrence joined ArQule as Executive Vice President and Chief Business Officer in April 2006. He was named Chief Operating Officer in October 2007 and President in April 2008. Previously he was at Pod Venture Partners, an international venture capital firm which he co-founded in 2001 and where he most recently served as general partner. He helped drive the strategic growth of that firm, including deal sourcing and structuring, syndication and business expansion activities. From 1991 to 2001, Mr. Lawrence was an attorney and partner at Mintz, Levin, Cohn, Ferris Glovsky and Popeo, P.C. While at Mintz Levin, he served as external corporate counsel to public and private companies, managed a transactional legal practice and provided strategic guidance to clients during periods of rapid growth and transformative corporate events. His public financing experiences include the initial public offering and other financings for America Online, Inc. (AOL), as well as public financings for Biogen, Human Genome Sciences, Hybridon and other prominent companies. Mr. Lawrence worked on numerous mergers and acquisitions, including Roche/Compuchem, AOL/Time Warner, Steinway Piano, DEC/Intel, and Mitotix/GPC Biotech. From 1989 to 1991, Mr. Lawrence worked in the Corporate Law Department of Gaston & Snow. He holds a Bachelor’s degree from Amherst College, and a J.D. from Boston University School of Law.

TABLE OF CONTENTS

Robert J. Weiskopf

Chief Financial Officer and Treasurer

Mr. Weiskopf joined ArQule in February 2007 as Vice President of Finance, Corporate Controller, and Treasurer and was promoted to Chief Financial Officer and Treasurer in May 2015. Prior to that, Mr. Weiskopf was Chief Financial Officer of Aware Inc. from 2004 until 2006 and Director of Finance at Lightbridge, Inc. from 2000 to 2004. He held a number of financial management positions of increasing responsibility at Digital/Compaq Computer Corporation for 19 years and began his career working at Ernst & Young LLP for five years. Mr. Weiskopf was also a part-time instructor in the Boston University M.B.A. program. Mr. Weiskopf is a Certified Public Accountant and holds a B.S.B.A. magna cum laude and M.S.B.A. in accounting from the University of Massachusetts at Amherst.

Brian Schwartz, M.D.

Chief Medical Officer

Dr. Schwartz joined ArQule in July 2008 from Ziopharm Oncology, Inc., where as Senior Vice President, clinical and regulatory affairs, and Chief Medical Officer he built and led clinical, regulatory, and quality assurance departments responsible for the development of new cancer drugs. Prior to Ziopharm, Dr. Schwartz held a number of positions at Bayer Healthcare. His experience in oncology has encompassed the clinical development of novel cytostatic, cytotoxic and immunological agents. At Bayer, Dr. Schwartz was a key physician responsible for the global clinical development of Nexavar® (sorafenib) and led the clinical team through a successful Phase 3 trial in renal cell cancer, leading to FDA approval. He has extensive regulatory experience working with the FDA's Oncology Division, the European Medicines Agency (EMA), and numerous other health authorities. Dr. Schwartz has also been responsible for U.S. clinical and regulatory activities, including Phase 4 studies and interactions with the National Cancer Institute and other oncology cooperative groups. Dr. Schwartz received his medical degree from the University of Pretoria, South Africa, practiced medicine, and worked at the University of Toronto prior to his career in industry.

ITEM 1A. RISK FACTORS

RISKS RELATED TO DEVELOPMENT, CLINICAL TESTING AND REGULATION OF OUR PRODUCTS CANDIDATES

Our product candidates are in preclinical and clinical stages of development and we may not successfully develop a product candidate that becomes a commercially viable drug.

The discovery and development of drugs is inherently risky and involves a high rate of failure. Our ability to generate revenue from product sales, which we do not expect will occur in the short term, if ever, will depend heavily on the successful development and eventual commercialization of one or more of our product candidates. We do not have extensive experience in discovery and development of commercial drugs. Our product candidates and drug research programs will continue to require significant, time-consuming and costly research and development, testing and regulatory approvals.

In addition to our clinical stage programs, we have a limited number of preclinical and research-stage programs in our pipeline. Despite our efforts, our product candidates may not:

- offer therapeutic or other improvement over existing competitive therapies;
- be proven safe and effective in clinical trials;
- meet applicable regulatory standards;
- be capable of being produced in commercial quantities at acceptable costs; and
- if approved for commercial sale, be successfully marketed as pharmaceutical products.

Our viability as a company may depend, in part, on our ability to create product candidates for ourselves and our collaborators and if we are unable to do so, our business will be harmed.

TABLE OF CONTENTS

We must show the safety and efficacy of our product candidates through expensive, time consuming preclinical testing and clinical trials, the results of which are uncertain and governed by exacting regulations.

Our product candidates are in clinical or preclinical stages of development and may not prove to be sufficiently safe or effective in more advanced human clinical trials. We will need to conduct extensive further testing of all of our product candidates, expend significant additional resources and possibly partner emerging programs to realize commercial value from any of our product candidates.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we and our collaboration partners must demonstrate through preclinical studies (laboratory or animal testing) and clinical trials (human testing) that our proposed products are safe and effective for use in each target indication. This testing is expensive and time-consuming, and failure can occur at any stage. If we terminate a preclinical or clinical program, we will have expended resources in an effort that will not provide a return on our investment and missed the opportunity to have allocated those resources to potentially more productive uses.

Our product candidates, as well as the activities associated with their research, development and commercialization, are subject to extensive regulation by the FDA in the United States and by comparable authorities outside of the United States, for example the European Medicines Agency in the European Union. These regulations govern the manufacturing, assessment of benefit and risk, safety, labeling, storage, commercialization and reimbursement of our product candidates. We or our collaboration partners may encounter problems in clinical trials that may cause us or the FDA or foreign regulatory agencies to delay, suspend or terminate our clinical trials at any phase. Changes in regulations or the process for regulatory review during the development or approval phases of our product candidates may cause development delays or rejection of an application for approval.

Non-U.S. jurisdictions have different approval procedures than those required by the FDA, and these jurisdictions may impose additional testing requirements for our product candidates. In addition, although the FDA may accept data from clinical trials conducted outside the United States, acceptance of this data is subject to conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and performed by qualified investigators in accordance with ethical principles. The trial population also must adequately represent the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials are subject to the applicable local laws, FDA acceptance of the data will depend on its determination that the trials also complied with all applicable U.S. laws and regulations. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and delay or permanently halt our development of the applicable product candidate.

It is part of our strategy to pursue clinical development to take advantage of available accelerated regulatory approval processes, such as fast track and/or breakthrough therapy designation. For example, the FDA granted fast track designation to miransertib for the treatment of PROS. There is no guarantee that our other product candidates will show the evidence predictive of clinical benefit necessary to qualify for such regulatory treatment. The receipt of such a designation for a product candidate does not guarantee a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA or comparable foreign regulatory authorities. In addition, even if one or more of our product candidates qualifies for fast track and/or breakthrough therapy designation, the FDA or comparable foreign regulatory authorities may later decide to withdraw such designation if it determines that the product candidate no longer meets the conditions for qualification.

Delays in clinical testing could result in increased costs to us and delay our ability to obtain regulatory approval and commercialize our product candidates.

Clinical trials typically take several years to complete. The duration and cost of clinical trials vary greatly depending on the phase of development and the nature, complexity, and intended use of the drug being tested. Even if the results of our clinical trials are favorable, the clinical trials of our product candidates may take significantly longer than expected to complete. Delays in the commencement or completion of clinical testing for any of our product candidates could significantly affect our product development costs and business plan.

TABLE OF CONTENTS

We may experience numerous unforeseen events during, or as a result of, the clinical trial process that could delay or prevent us from receiving regulatory approval or commercializing our product candidates, including the following:

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or may require discussions regarding the scope or design of our clinical trials;

- our clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical and/or preclinical testing or to provide additional information about formulation or manufacture of our product candidates or clinical trial design or to abandon programs;

- we may experience delays related to reaching agreement on acceptable terms with prospective contract research organizations (CROs), clinical investigators and trial sites;

- we may be unable to source suitable diagnostic tests for our trials in targeted patient populations;

- we may be unable to manufacture or obtain sufficient quantities of a product candidate for use in clinical trials;

- failure by us, our employees, our CROs or their employees to conduct a clinical trial in accordance with all applicable FDA or other regulatory requirements or our clinical protocols;

- failure of our CROs or other third-party contractors to comply with all contractual requirements or to perform their services in a timely or acceptable manner;

- enrollment in our clinical trials for our product candidates may be slower than we anticipate;

- we, or regulators, may suspend or terminate our clinical trials if the participating patients are being exposed to unacceptable health risks;

- changes in applicable regulatory requirements and guidance may occur, which could require us to amend clinical trial protocols;

- the effects of our product candidates on patients may not have the desired therapeutic result or may have undesirable side effects that could delay or preclude regulatory approval or limit their commercial use, if approved; and

- lack of adequate funding to continue the clinical trial or the cost of clinical trials of our product candidates may be greater than we anticipate.

If we experience delays in completion of, or if we terminate, any of our clinical trials, the commercial prospects for our product candidates may be harmed, which may have a material adverse effect on our business, results of

operations, financial condition and prospects.

The results of early-stage clinical trials and preclinical studies may not be predictive of future results. Initial success in clinical trials may not be indicative of results obtained when these trials are completed or in later stage trials.

The results from initial preclinical studies and clinical trials of products under development are not necessarily indicative of results that will be obtained from subsequent or more extensive preclinical studies and clinical testing in humans. Clinical trials may not demonstrate sufficient safety and efficacy to obtain the required regulatory approvals or result in marketable products. Failure to adequately demonstrate the safety and efficacy of a product under development will delay and could prevent its regulatory approval.

A number of companies in the pharmaceutical industry, including ArQule and other biotechnology companies, have suffered significant setbacks in advanced clinical trials, even after generating promising results in earlier trials. For example, a positive randomized Phase 2 trial for tivantinib in hepatocellular carcinoma (HCC) did not lead to a positive outcome in our Phase 3 trials in HCC. In addition, from time to time we report interim data from our clinical trials. Interim data from a clinical trial may not be predictive of final results from the clinical trial.

TABLE OF CONTENTS

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

At any time, a clinical trial can be placed on "clinical hold" or temporarily or permanently stopped for a variety of reasons, principally for safety concerns. If any product candidates we develop are associated with serious adverse events or undesirable side effects we may need or regulatory authorities may require us to abandon their development or limit development to certain uses or subpopulations in which the adverse events or undesirable side effects are less prevalent, less severe, or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

Completion and duration of clinical trials depends on, among other things, our ability to enroll a sufficient number of patients, which is a function of many factors, including:

- the incidence among the general population of diseases which contain therapeutic endpoints chosen for evaluation;
- the eligibility criteria defined in the protocol;
- the size of the patient population required for analysis of the trial's therapeutic endpoints;
- our ability to recruit clinical trial investigators and sites with the appropriate competencies and experience;
- our ability to obtain and maintain patient consents; and
- competition for patients by clinical trial programs for other treatments.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would harm our financial position.

Failure to successfully validate, develop and obtain regulatory approval for companion diagnostics could harm our drug development strategy.

One of the key elements of our clinical development strategy is to seek to identify patient subsets within a disease category that may derive particular benefit from the product candidates we are developing. We seek to develop companion diagnostics to help us to identify patients within a particular subset, both during our clinical trials and in connection with the potential commercialization of our product candidates. Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as medical devices and require separate regulatory approval prior to commercialization. We do not develop companion diagnostics internally and thus we are dependent on the sustained cooperation and effort of third-party collaborators in developing and obtaining approval for these companion diagnostics. We, our partners and our companion diagnostic collaborators may encounter difficulties in developing and obtaining approval for companion diagnostics, including issues relating to selectivity/specificity, analytical validation, reproducibility, clinical validation or concordance. Any delay or failure by our collaborators to develop or obtain regulatory approval of a companion diagnostic could delay or prevent approval of our product candidates. In addition, our collaborators may encounter production difficulties or difficulties sourcing key materials that could constrain the supply of the companion diagnostic, and both they and we may have difficulties gaining acceptance of the use of the companion diagnostic in the clinical community. If such companion diagnostic were to fail to gain market acceptance, it would have an adverse effect on our ability to derive revenues

from sales of products that obtain marketing approval. In addition, the diagnostic companies with whom we and our partners work may decide to discontinue selling or manufacturing the companion diagnostic (or components thereof) that we anticipate using in connection with development and commercialization of our product candidates, or our relationship with such diagnostic companies may otherwise terminate according to the terms of our agreements with them. We may not be able to enter into

TABLE OF CONTENTS

arrangements with another diagnostic company to obtain supplies of alternative diagnostic tests for use in connection with the development and commercialization of our product candidates or do so on commercially reasonable terms. In addition, many current companion diagnostic products use immunohistochemistry (IHC) to identify patients within a target group. The results of IHC tests are determined by pathologists and clinicians and therefore are subject to variation from reader to reader. While efforts are made to ensure rigorous training, such inherent variability can impact patient selection and cause variation from lab to lab and trial to trial.

We have limited clinical development and commercialization experience.

We have limited experience conducting clinical trials and have never obtained regulatory approvals for any drug. We have not independently completed a Phase 3, or pivotal, clinical trial, filed an NDA or commercialized a drug. We have no experience as a company in the sale, marketing or distribution of pharmaceutical products and do not currently have a sales and marketing organization. Developing commercialization capabilities will be expensive and time-consuming, and could delay any product launch. We may not be able to develop a successful commercial organization. To the extent we are unable or determine not to acquire these resources internally, we will be forced to rely on third-party clinical investigators, clinical research organizations, marketing organizations or our collaboration partners as we did for our Phase 3 trial in HCC. If we are unable to establish adequate capabilities independently or with others, our drug development and commercialization efforts could fail, and we may be unable to generate product revenues.

If our drug discovery and development programs do not progress as anticipated, our revenue and stock price could be negatively impacted.

We estimate the timing of a variety of preclinical, clinical, regulatory and other milestones for planning purposes, including when a product candidate is expected to enter clinical trials, how soon patients will be recruited and enrolled in these trials, when a clinical trial will be completed and when an application for regulatory approval will be filed. We base our estimates on facts that are currently known to us and on a variety of assumptions, many of which are beyond our control. If we or our collaborators do not achieve milestones when anticipated our revenues and stock price could decline. In addition, our research and clinical testing may be delayed or abandoned if we or our competitors subsequently discover other compounds that show improved safety or efficacy compared to our product candidates, which could limit our ability to achieve milestones and the associated revenue when anticipated or at all, cause us to incur additional expense and cause the market price of our common stock to decline.

Even if our product candidates obtain regulatory approval, we and our collaborators will be subject to ongoing government regulation.

Even if regulatory authorities approve any of our product candidates, the manufacture, marketing and sale of these drugs will be subject to strict and ongoing regulation. Compliance with such regulations may consume substantial financial and management resources and expose us and our collaborators to the potential for other adverse circumstances. For example, a regulatory authority can place restrictions on the sale or marketing of a drug in order to manage the risks identified during clinical trials or after the drug is on the market. A regulatory authority can condition the approval of a drug on costly post-marketing follow-up studies. Based on these studies, if a regulatory authority does not believe that the drug demonstrates a clinical benefit to patients or an acceptable safety profile, it could limit the indications for which a drug may be sold or revoke the drug’s marketing approval. In addition, identification of certain side effects either during clinical trials or after a drug is on the market may result in reformulation of a drug, additional preclinical and clinical trials, labeling changes, implementation of a Risk Evaluation and Mitigation Strategy, termination of ongoing clinical trials or withdrawal of approval. Any of these events could delay or prevent us from generating revenue from the commercialization of these drugs and cause us to incur significant additional costs.

In addition, if we fail to comply with applicable requirements, we may be subject to various regulatory, civil, or criminal sanctions, including Warning or Untitled Letters, withdrawal or suspension of product approvals. FDA refusal to approval new applications, consent decrees, injunctions, product seizures or detentions, and civil and criminal penalties.

TABLE OF CONTENTS

Even if we or our collaborators bring products to market, we may be unable to price our products effectively or obtain adequate reimbursement for sales of our products, which would have an adverse effect on our revenues.

The requirements governing product licensing, pricing and reimbursement 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 product-licensing approval is granted. As a result, we may obtain regulatory approval for a drug candidate in a particular country, but then be subject to price regulations that reduce our profits from the sale of the product. In some foreign markets pricing of prescription pharmaceuticals is subject to continuing government control after initial marketing approval. Varying price regulation between countries can lead to inconsistent prices and some re-selling by third parties of products from markets where products are sold at lower prices to markets where those products are sold at higher prices. Any practice of exploiting price differences between countries could undermine our sales in markets with higher prices and reduce the sales of our future products, if any.

Additionally, third party payors, such as government and private insurance plans, frequently require companies to provide rebates and predetermined discounts from list prices and are increasingly challenging the prices charged for pharmaceuticals and other medical products. Our products may not be considered cost-effective, and reimbursement to the patient may not be available or be sufficient to allow the sale of our products on a competitive basis. We, or our collaborators, may not be able to negotiate favorable reimbursement rates for our products. If we, or our collaborators, fail to obtain an adequate level of reimbursement for our products by third-party payors, sales of the drugs would be adversely affected or there may be no commercially viable market for the products.

RISKS RELATED TO OUR FINANCIAL CONDITION

We have incurred significant losses since our inception and anticipate that we will incur significant continued losses for the next several years, and our future profitability is uncertain.

From our inception in 1993 through December 31, 2018 we have incurred cumulative losses of approximately $548 million. These losses have resulted principally from the costs of our research and development activities, acquisitions, and enhancements to our technology. In the past we derived our revenue primarily from license and technology transfer fees and payments for compound deliveries associated with our discontinued chemistry services operations, research and development funding paid under our agreements with collaboration partners, and from milestone payments under collaboration agreements.

We expect that we will continue to incur significant expenses in order to fund research, development and commercialization of our product candidates. Our expenses could increase beyond our expectations if we are required by the FDA or other regulatory authorities to perform trials in addition to those that we currently expect, or if there are any delays in completing our clinical trials or the development of any of our product candidates. We currently have a number of product candidates in various stages of clinical development. As a result, we will need to generate significant additional revenues to achieve profitability.

To attain profitability, we will need to develop clinical products successfully and market and sell them effectively, either by ourselves or with collaborators. We have never generated revenue from the commercialization of our product candidates, and there can be no guarantee that we will be able to do so. Even if were to generate product revenues and achieve profitability, we may not be able to sustain or grow profitability. Because of the numerous risks and uncertainties associated with the development of drugs, we are unable to predict the extent of any future losses or when we will become profitable, if at all. If we fail to become profitable, or if we are unable to fund our continuing losses, we may be unable to continue our business.

We are party to a loan and security agreement that contains operating and financial covenants that may restrict our business and financing activities.

In 2017, we entered into a loan and security agreement with Oxford Finance LLC for a term loan to us in the principal amount of $15 million. We are required to repay the loan on or before August 1, 2022. Borrowings under this loan and security agreement are secured by substantially all of our assets, excluding certain intellectual property rights. The loan and security agreement restricts our ability, among other things, to:

TABLE OF CONTENTS

- sell, transfer or otherwise dispose of any of our business or property, subject to limited exceptions;
- make material changes to our business or management team;
- enter into transactions resulting in significant changes to the voting control of our stock;
- make certain changes to our organizational structure;
- consolidate or merge with other entities or acquire other entities;
- incur additional indebtedness or create encumbrances on our assets;
- pay dividends, other than dividends paid solely in shares of our common stock, or make distributions on and, in certain cases, repurchase our stock;
- repay subordinated indebtedness;
- enter into certain exclusive licensing arrangements; or
- make certain investments.

In addition, we are required under our loan agreement to comply with various affirmative operating covenants. The operating covenants and restrictions and obligations in our loan and security agreement, as well as any future financing agreements that we may enter into, may restrict our ability to finance our operations, engage in business activities or expand or fully pursue our business strategies. Our ability to comply with these covenants may be affected by events beyond our control, and we may not be able to meet those covenants. A breach of any of these covenants could result in a default under the loan and security agreement, which could cause all of the outstanding indebtedness under the facility to become immediately due and payable and eliminate our eligibility to receive additional loans under the agreement.

If we are unable to generate sufficient cash available to repay our debt obligations when they become due and payable, either when such obligations become due, when they mature, or in the event of a default, we may not be able to obtain additional debt or equity financing on favorable terms, if at all, which may negatively impact our business operations and financial condition.

We may need substantial additional funding and we may be unable to raise capital when needed, or on terms favorable to us, which could force us to delay, reduce or eliminate our drug discovery, product development and commercialization activities.

Developing drugs, conducting clinical trials, and commercializing products are expensive. Our operations have consumed substantial amounts of cash since inception, and we expect to continue to spend substantial amounts to continue our preclinical and clinical development programs. We anticipate that our cash, cash equivalents and marketable securities on hand at December 31, 2018, financial support from our licensing agreements and the one year

extension of our loan agreement will be sufficient to finance our operations for at least the next 12 months. Our future funding requirements will depend on many factors, including:

- the progress and cost of our ongoing and future clinical trials and other research and development activities;
- our ability to establish and maintain additional collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under our license agreements with Basilea and Sinovant;
- the costs and timing of obtaining regulatory approvals;
- the costs of filing, prosecuting, maintaining, defending and enforcing all patent applications, claims, patents and other intellectual property rights;
- the cost and timing of securing manufacturing capabilities for our clinical product candidates and commercial products, if any;

TABLE OF CONTENTS

- the costs and timing of commercializing our product candidates, including establishing or contracting for sales, marketing and distribution capabilities, if any such product candidate receives regulatory approval for commercial sale; and

- the costs of any acquisitions of or investments in businesses, products or technologies.

We may seek the capital necessary to fund our operations through public or private equity offerings, debt financings, or collaboration and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted and the terms of such securities may include liquidation or other preferences that adversely affect our stockholders' rights. Other debt-financing arrangements may require us to pledge certain assets and enter into covenants that would restrict certain business activities or our ability to incur further indebtedness. If we raise additional funds through collaboration and licensing arrangements with third parties, we may have to relinquish rights to certain product candidates that we might otherwise seek to develop or commercialize independently, or grant licenses on terms that are not favorable to us. There can be no assurance that sufficient funds will be available to us when required, on satisfactory terms, or at all. If we are unable to obtain additional funds when needed, we may have to delay, reduce the scope of or eliminate some of our development and commercialization programs, or obtain funds through other arrangements on unattractive terms, which could prevent us from successfully executing our business strategy.

We have federal and state net operating loss carryforwards ("NOLs") and research and development credit carryforwards which, if we were to become profitable, could be used to offset/defer federal and state income taxes. Such carryforwards may not, under certain circumstances related to changes in ownership of our stock, be available to us.

As of December 31, 2018, we had federal NOL, state NOL, and research and development credit carryforwards of approximately $422 million, $241 million and $28 million respectively, which expire at various dates through 2037. Out of the total federal NOL of $422 million $12.6 million of NOL generated in 2018 has an indefinite life. Such carryforwards could potentially be used to offset certain future federal and state income tax liabilities. Utilization of carryforwards may be subject to a substantial annual limitation pursuant to Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, as well as similar state provisions due to ownership changes that have occurred previously or that could occur in the future. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50 percentage points over a three-year period. We undertook a detailed study of our NOLs and research and development credit carryforwards through January 31, 2019 to determine whether such amounts are likely to be limited by Sections 382 or 383. As a result of this analysis, we currently do not believe any Section 382 or 383 limitations will significantly impact our ability to offset income with available NOLs and research and development credit carryforwards. However, future ownership changes under Section 382 may limit our ability to fully utilize these tax benefits. Any limitation may result in expiration of a portion of the carryforwards before utilization. If we are not able to utilize our carryforwards, we would be required to use our cash resources to pay taxes that would otherwise have been offset, thereby reducing our liquidity.

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

In December 2017, the United States enacted the Tax Cuts and Jobs Act of 2017 ("TCJA"), which significantly changed the U.S. federal income tax system. The TCJA, among other things, significantly changed corporate taxation, including reducing the U.S. federal corporate tax rate from a top marginal tax rate of 35% to a flat rate of 21%, limiting the tax deduction for net business interest expense to 30% of adjusted taxable income (with certain excepted businesses), limiting the deduction for NOLs generated during or after 2018 to 80% of annual taxable income and eliminating NOL carrybacks, and modifying or repealing many business deductions and credits (including reducing the business tax credit for certain clinical testing expenses incurred in the testing of certain drugs for rare diseases or conditions generally referred to as "orphan drugs"). We continue to examine the impact the TCJA may have on our business.

28

TABLE OF CONTENTS

RISKS RELATED TO COLLABORATIONS

Part of our business strategy involves collaborative out-licensing of our product candidates. We may not be able to find collaborators or successfully form suitable collaborations to further our drug development and commercialization efforts.

We have sought and may seek collaborators for our drug development and commercialization efforts. For example, in April 2018 we exclusively licensed derazantinib (ARQ 087) to Basilea Pharmaceutica Limited in the United States, European Union, Japan and the rest of the world, excluding the People's Republic of China, Hong Kong, Macau, and Taiwan where derazantinib was exclusively licensed to Sinovant Sciences Ltd., a subsidiary of Roivant Sciences Ltd. in February 2018. We may seek to enter into additional future collaborations to obtain external financing for drug development and to obtain access to drug development and commercialization expertise.

We face significant competition in seeking appropriate collaborators. This competition is particularly intense in the oncology field. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors, including:

- the design or results of clinical trials;
- thee likelihood of approval by the FDA or similar regulatory authorities outside the United States;
- the potential market for the subject product candidate;
- the costs and complexities of manufacturing and delivering such drug candidate to patients;
- the existence of uncertainty with respect to our ownership of the applicable intellectual property, which can exist if there is a challenge to such ownership without regard to the merits of the challenge;
- the potential of competing products; and
- industry and market conditions generally.

Even if we are able to attract the interest of potential collaborators, the negotiation, documentation and implementation of collaboration agreements are complex and time-consuming. Collaborations may not be available on commercially acceptable terms and, if formed, may not be commercially successful or, if successful, may not realize sufficient benefit for us. If we are unable to form collaborations, we may not gain access to the financial resources and industry expertise necessary to develop and commercialize product candidates or successfully market any product candidate we develop on our own and, therefore, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs. In addition, our past, existing and future collaboration terms contain or will likely contain limitations on the areas of research and development that we may pursue, either alone or in collaboration with third parties.

Our success depends in part on the efforts of our current and possible future collaborators, who will likely have substantial control and discretion over the continued development and commercialization of product candidates that are the subjects of our collaborations.

Our current collaborators and future collaborators will have significant discretion in determining the efforts and amount of resources that they dedicate to our collaborations. Our collaborators may determine not to proceed with clinical development or commercialization of a particular product candidate for a number of reasons that are beyond our control, even under circumstances where we might have continued such program. In addition, our rights to receive milestone payments and royalties from our collaborators will depend on our collaborators' abilities to establish the safety and efficacy of our product candidates, obtain regulatory approvals and achieve market acceptance of products developed from our product candidates. We will also depend on our collaborators to manufacture clinical, and possibly, commercial quantities of our product candidates. Our collaborators may not be successful in manufacturing our product candidates or successfully commercializing them.

TABLE OF CONTENTS

We face additional risks in connection with our existing and future collaborations, including the following:

- our collaborators may develop and commercialize, either alone or with others, products that are similar to or competitive with the products that are the subject of the collaboration with us;

- our collaborators may underfund, not commit sufficient resources to, or conduct in an unsatisfactory matter the testing, development, manufacturing, or commercialization of our product candidates;

- our collaborators may not properly maintain or defend our intellectual property rights or may utilize our proprietary information in such a way as to invite litigation that could jeopardize or potentially invalidate our intellectual property or proprietary information or expose us to potential liability;

- our collaborators may encounter conflicts of interest, changes in business strategy or other business issues which could adversely affect their willingness or ability to fulfill their obligations to us. For example, pharmaceutical and biotechnology companies historically have re-evaluated their priorities following mergers and consolidations, which have been common in recent years;

- disputes may arise between us and our collaborators delaying or terminating the research, development or commercialization of our product candidates, resulting in significant litigation or arbitration that could be time-consuming and expensive, or causing collaborators to act in their own self-interest and not in the interest of our stockholders;

- we might not have the financial or human resources to meet our obligations or take advantage of our rights under the terms of our existing and future collaborations; and

- our existing collaborators may exercise their respective rights to terminate without cause their collaborations with us, in which event, we might not be able to complete development and commercialization of the affected product candidates on our own.

RISKS RELATED TO RELATIONSHIPS WITH THIRD PARTY VENDORS

We rely heavily on third parties such as contract research organizations, to conduct our nonclinical studies and clinical trials. If third parties upon which we rely do not perform as contractually required or expected, we may not be able to develop further, obtain regulatory approval for or commercialize our product candidates.

We do not have the ability or the human resources to perform all of the testing or conduct all of the clinical trials that are necessary in connection with the development of our product candidates. As a result, we rely on CROs, clinical data management organizations, medical institutions, and clinical investigators to ensure the proper and timely conduct of both our our clinical trials and nonclinical studies. While we have agreements governing their activities, we have limited influence over their actual performance. Nevertheless, we are responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on CROs does not relieve us of our responsibilities. We and our CROs are required to comply with the FDA's current Good Clinical Practices (GCP), which are regulations and guidelines enforced by the FDA for all of our product candidates in clinical development. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform

additional clinical trials before approving our marketing applications. In addition, our agreements with these third parties, including CROs, might terminate for a variety of reasons. If we need to enter into alternative arrangements, that would delay our product development activities.

We have a limited ability to control whether or not our CROs devote sufficient time and resources to our ongoing clinical and nonclinical programs. These CROs may also have relationships with other entities, including our competitors, for whom they may also be conducting clinical studies. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may allow our potential competitors to access our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of

TABLE OF CONTENTS

the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates.

We rely on third parties to manufacture sufficient quantities of our product candidates to conduct preclinical and clinical studies. We may not be able to maintain these relationships and could experience supply disruptions outside of our control.

To date, our product candidates have been manufactured in relatively small quantities for preclinical and clinical trials. We have no experience in manufacturing any of our product candidates on a large scale and have contracted with third party manufacturers (as have our collaborators) to provide material for clinical trials and to assist in the development and optimization of our manufacturing processes and methods. Our and our collaborators' ability to conduct clinical trials and commercialize our product candidates will depend on the ability of such third parties to manufacture our products on a large scale at a competitive cost and in accordance with the FDA's current Good Manufacturing Practices (GMP) and other regulatory requirements. Significant scale-up of manufacturing may result in unanticipated technical challenges and may require additional validation studies that the FDA must review and approve.

We do not currently have arrangements in place for a second source for certain aspects of our supply chain. If our current contract manufacturers cannot perform as agreed or if they experience a shutdown or disruption in the facilities used to produce the applicable material, due to technical, regulatory or other problems, we may be required to replace such manufacturer. Although we believe that there are several potential alternative manufacturers at each step of our supply chain, we may incur added costs and delays in identifying and qualifying any such replacement manufacturer and in reaching agreement with such alternative manufacturer. If we or our collaborators are not able to obtain contract manufacturing on commercially reasonable terms, obtain or develop the necessary materials and technologies for manufacturing, or obtain intellectual property rights necessary for manufacturing, we may not be able to conduct or complete clinical trials or commercialize our product candidates.

Our product candidates require precise, high-quality manufacturing. The failure to achieve and maintain these high manufacturing standards, including the incidence of manufacturing errors, could result in patient injury or death, clinical holds, product recalls or withdrawals, delays or failures in product testing or delivery, cost overruns or other problems that could seriously hurt our business.

The facilities used by our contract manufacturers may undergo inspections by the FDA or comparable foreign regulatory authorities for compliance with GMP regulations or their foreign equivalent. We and our collaborators have limited control over our contract manufacturers' compliance with these regulations and standards. If these facilities do not satisfy GMP requirements, we and our collaborators may need to conduct additional validation studies, or find alternative manufacturing facilities, either of which would result in significant cost to us as well as a delay in obtaining approval for any affected product candidate. Failure by such third-party manufacturers to comply with applicable regulations could also result in sanctions being imposed (including fines, injunctions and civil penalties), clinical holds, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecution.

RISKS RELATED TO COMPETITION

The drug research and development industry is highly competitive, and we compete with some companies that have a broader range of capabilities and better access to resources than we do.

The pharmaceutical and biotechnology industries are characterized by rapid and continuous technological innovation. We compete with companies worldwide that are engaged in the research and discovery, licensing, development and commercialization of drug candidates, including, in the area of small molecule anti-cancer therapeutics, companies such as AbbVie Inc., Amgen, Inc., Ariad Pharmaceuticals, Inc., Astellas Pharma, Inc., Array BioPharma Inc., AstraZeneca PLC, Celgene Corporation, Curis, Inc.,

TABLE OF CONTENTS

Exelixis, Inc., Eli Lilly and Company, FORMA Therapeutics, Gilead Sciences, Inc., GlaxoSmithKline plc, Incyte Corporation, Infinity Pharmaceuticals, Inc., Johnson and Johnson, Merck, Merck KGaA, Novartis AG, Pfizer, Inc., Principia Biopharma, Inc., the Roche Group, Sunesis Pharmaceuticals, Inc., Takeda Pharmaceuticals Co. Ltd., and many others.

With respect to ARQ 531, we are aware of a number of companies that are or may be pursuing different approaches to C481S-mutant BTK inhibition, including Aptose Biosciences Inc., Roche and Sunesis Pharmaceuticals. Moreover, numerous companies are also pursuing inhibitors of wild-type BTK, including AbbVie with its drug, IMBRUVICA, and AstraZeneca with its drug, CALQUENCE. Other companies with BTK inhibitors currently in development include Astra Zeneca, BeiGene Co., Ltd., Merck KGaA, Eli Lilly, Gilead, GlaxoSmithKline, Principia Biopharma and others. Other approved drugs that may compete to treat ibrutinib refractory patients, including patients with C481S-mutant BTK, include AbbVie's Bcl-2 inhibitor, VENCLEXTA, and Verastem Oncology's COPIKTRA.

With respect to miransertib (ARQ 092) in rare diseases, we believe Roche is developing taselisib for PIK3CA-Related Overgrowth Syndromes (PROS). Regarding miransertib in oncology, we are aware of a number of companies that are or may be pursuing different approaches to AKT inhibition, including Astra Zeneca, Bayer, Eli Lilly, Merck, Novartis, Rexahn Pharmaceuticals, Inc. and Roche. Moreover, numerous companies have pursued and are pursuing inhibitors of PI3K and mTOR, two kinases in the PI3K-AKT-mTOR pathway; these drugs include Idelalisib, an approved PI3K inhibitor, and Everolimus, Temsirolimus and Rapamycin, approved mTOR inhibitors.

With respect to derazantinib (ARQ 087), we are aware of a number of companies that are or may be pursuing a number of different approaches to FGFR inhibition, including Astra Zeneca, Bayer, BioClin Therapeutics, Debiopharm Group, Boehringer Ingelheim International GmbH, Eisai Co., Ltd., Five Prime Therapeutics, Incyte, Johnson & Johnson, Novartis, Pfizer, Principia Biopharma, Servier and Taiho Oncology. With respect to iCCA, our lead indication for ARQ 087, we are aware of a number of companies with products under development, including Agios Pharmaceuticals, Inc., Bayer Healthcare Pharmaceutical, Bristol-Myers Squibb, Cellact Pharma Gmbh, Concordia Healthcare, Dainippon Sumitomo Pharma Co., Ltd., Delcath Systems, Inc., Exelixis, InCyte, Novartis, Oncotherapy Services, Inc. and Spectrum Pharmaceuticals, Inc.

Even if we are successful in bringing products to market, we face substantial competitive challenges in effectively marketing and distributing our products. Companies and research institutions, including large pharmaceutical companies and biotechnology companies with significantly much greater financial resources and more experience in developing products, conducting clinical trials, obtaining FDA and foreign regulatory approvals and bringing new drugs to market are developing products within the field of oncology. Some of these entities already have competitive products on the market or product candidates in more advanced stages of development than we do. By virtue of having or introducing competitive products on the market before us, these entities may gain a competitive advantage. In addition, there may be product candidates of which we are not aware at an earlier stage of development that may compete with our product candidates. Some of our competitors have entered into collaborations with leading companies within our target markets.

We are in a rapidly evolving field of research. Consequently, our technology may be rendered non-competitive or obsolete by approaches and methodologies discovered by others, both before and after we have gone to market with our products. We also face competition from existing therapies that are currently accepted in the marketplace and from the impact of adverse events in our field that may affect regulatory approval or public perception.

We anticipate that we will face increased competition in the future as new companies enter the market and advanced technologies become available. If we are unable to successfully compete in our chosen field, we will not become profitable.

We may not be able to recruit and retain the scientists and management we need to compete.

Our success depends on our ability to attract, retain and motivate highly skilled scientific personnel and management, and our ability to develop and maintain important relationships with leading academic institutions, clinicians and scientists. We are highly dependent on our senior management and scientific

TABLE OF CONTENTS

staff, and the loss of the services of one or more of our other key employees could have an adverse effect on the successful completion of our clinical trials or the commercialization of our product candidates.

We compete intensely with pharmaceutical and biotechnology companies, including our collaborators, medicinal chemistry outsourcing companies, contract research and manufacturing organizations, and academic and research institutions in the recruitment of scientists and management. The shortage of personnel with experience in drug development could lead to increased recruiting, relocation and compensation costs, which may exceed our expectations and resources. If we cannot hire additional qualified personnel, the workload may increase for both existing and new personnel. If we are unsuccessful in our recruitment efforts, we may be unable to execute our strategy.

RISKS RELATED TO INTELLECTUAL PROPERTY

Our patents may fail to protect our business. If we are unable to obtain and maintain patent protection for our product candidates or if the scope of the patent protection obtained is not sufficiently broad, third parties may be able to develop and commercialize technology and products similar or identical to ours, which could adversely affect our ability to compete in the market.

To be successful and compete, we must obtain and maintain patents on our products and technology. Where appropriate, we seek patent protection for certain aspects of the technology we are developing, but patent protection may not be available for some of our product candidates or their use, synthesis or formulations. The patent position of biotechnology firms is highly uncertain, involves complex legal and factual questions, and has recently been the subject of much litigation. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until substantially after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patent applications, or that we were the first inventors to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. In addition, we may rely on our third-party collaborators to file patent applications and prosecute patents relating to proprietary technology that we develop as a part of the collaboration. If our present or future collaborators fail to file and prosecute all necessary and desirable patent applications in a timely manner, our business may be adversely affected.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. For example, the Leahy-Smith America Invents became fully effective in March 2013 and included, among other things, moving to a first inventor-to-file system. Additionally, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. While we cannot predict what form any new patent reform regulations ultimately may take, final governmental rule-making and case law interpreting the new statute could introduce new substantive rules, procedures and case law bases for challenging patents, and certain reforms that make it easier for competitors to challenge our patents could have a material adverse effect on our business.

We do not know whether our patent applications will result in issued patents. The standards that the United States Patent and Trademark Office (USPTO) and its foreign counterparts use to grant patents are not always applied predictably or uniformly and can change. In addition, the receipt of a patent might not provide much practical protection. If we receive a patent with a narrow scope it will be easier for competitors to design products that do not infringe our patent. We cannot be certain that we will receive any additional patents, that the claims of our patents will offer significant protection for our technology, or that our patents will not be challenged, narrowed, invalidated or circumvented.

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

TABLE OF CONTENTS

and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We only have limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. The laws of some foreign countries do not protect intellectual property rights to the same extent as the laws of the United States. Many companies have encountered significant problems in protecting and defending such rights in foreign jurisdictions. Moreover, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets or other forms of intellectual property, which could make it difficult for us to prevent competitors in some jurisdictions from marketing competing products in violation of our proprietary rights. 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 the patent. In some jurisdictions, compulsory licensing laws compel patent owners to grant licenses to third parties. Such compulsory licenses could be extended to include some of our product candidates, which could limit our potential revenue opportunities.

The drug research and development industry has a history of patent and other intellectual property litigation, and we may be involved in costly intellectual property lawsuits.

The drug research and development industry has a history of patent and other intellectual property litigation, and we believe these lawsuits are likely to continue. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference, derivation, reexamination or inter partes review proceedings before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Because patent applications can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that we infringe with our product candidates or resulting products. In addition, technology created under our research and development collaborations may infringe the intellectual property rights of third parties, in which case we may not receive milestone or royalty revenue from those collaborations.

If we do not prevail in an infringement lawsuit brought against us, we might have to pay substantial monetary damages, and we could be required to stop the infringing activity or obtain a license to use the patented technology or redesign our products so as not to infringe the patent. We may not be able to enter into licensing arrangements at a reasonable cost or effectively redesign our products. Any inability to secure licenses or alternative technology could delay the introduction of our products or prevent us from manufacturing or selling products. Even if resolved in our favor, litigation relating to intellectual property claims may cause us to incur significant expenses and could distract management from their normal responsibilities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

TABLE OF CONTENTS

Agreements we have with our employees, consultants and collaborators may not afford adequate protection for our trade secrets, confidential information and other proprietary information.

In addition to patent protection, we also rely on trade secrets and know-how. To protect our trade secrets and know-how, we require our employees, consultants and advisors to execute agreements regarding the confidentiality and ownership of such proprietary information. We cannot guarantee, however, that these agreements will provide us with adequate protection against improper use or disclosure of confidential information, and there may not be adequate remedies in the event of unauthorized use or disclosure. Our employees, consultants or advisors may unintentionally or willfully disclose our information to competitors. In addition, in some situations, these agreements may conflict with, or be subject to, the rights of third parties with whom our employees, consultants or advisors had or have previous employment or consulting relationships. Like patent litigation, enforcing a claim that a third party illegally obtained and is using our trade secrets is expensive and time-consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing than our federal and state courts to protect trade secrets. Furthermore, others may independently develop substantially equivalent knowledge to that covered by our trade secrets and know-how. Our failure or inability to protect our proprietary information and techniques may inhibit or limit our ability to compete effectively or exclude certain competitors from the market.

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

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

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

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

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

A third party may hold intellectual property, including patent rights, that are important or necessary to the development of one or more of our product candidates. It may be necessary for us to use the patented or proprietary technology of a third party to commercialize our own technology or product candidates, in which case we would be required to obtain a license from such third party. A license to such intellectual property may not be available or may not be available on commercially reasonable terms, which could have a material adverse effect on our business and financial condition.

The licensing and acquisition of third-party intellectual property rights is a competitive practice, and companies that may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their larger size and cash resources or greater clinical development and commercialization capabilities. We may not be able to successfully complete such negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to acquire.

TABLE OF CONTENTS

Obtaining and maintaining our patent protection depends on compliance with various procedural, documentary, fee payment and other requirements imposed by governmental patent offices, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

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

If we are not able to obtain patent term extensions in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for our product candidates, our business may be impaired.

Product candidates we develop that are approved for commercial marketing by the FDA may be subject to the provisions of the Drug Price Competition and Patent Term Restoration Act of 1984, known as the “Hatch-Waxman Act.” The Hatch-Waxman Act provides companies with marketing exclusivity for varying time periods during which generic versions of a drug may not be marketed and allows companies to apply to extend patent protection for up to five additional years. Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request. If we are unable to obtain patent term extension, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration sooner, and our revenue would likely be reduced.

RISKS RELATED TO LEGAL AND COMPLIANCE

We face potential liability related to the privacy of health information.

Most health care providers, including research institutions from which we or our collaborators obtain patient information, are subject to privacy regulations promulgated under the Health Insurance Portability and Accountability Act of 1996, or HIPAA. Although we are not directly regulated by HIPAA, we could face substantial criminal penalties if we knowingly receive individually identifiable health information from a health care provider or research institution that has not satisfied HIPPA’s disclosure standards. In addition, certain state privacy laws may apply directly to our operations and/or those of our collaborators and may impose restrictions on our use and dissemination of individuals’ health information. Moreover, patients about whom we or our collaborators obtain information, as well as the providers who share this information with us, may have contractual rights that limit our ability to use and disclose the information. Claims that we have violated individuals’ privacy rights or breached our contractual obligations, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

The collection and use of personal health data in the European Union had previously been governed by the provisions of the Data Protection Directive, which has been replaced by the General Data Protection Regulation (GDPR) which became effective on May 25, 2018. While the Data Protection Directive did not apply to organizations based outside the EU, the GDPR has expanded its reach to include any business, regardless of its location, that provides goods or services to residents in the EU. This expansion would incorporate our clinical trial activities in EU member states. The GDPR imposes strict requirements on controllers and processors of personal data, including special protections for “sensitive information” which

TABLE OF CONTENTS

includes health and genetic information of data subjects residing in the EU. GDPR grants individuals the opportunity to object to the processing of their personal information, allows them to request deletion of personal information in certain circumstances, and provides the individual with an express right to seek legal remedies in the event the individual believes his or her rights have been violated. Further, the GDPR imposes strict rules on the transfer of personal data out of the EU to the United States or other regions that have not been deemed to offer "adequate" privacy protections. Failure to comply with the requirements of the GDPR and the related national data protection laws of the EU member states, which may deviate slightly from the GDPR, may result in significant fines.

There is significant uncertainty related to the manner in which data protection authorities will seek to enforce compliance with GDPR. For example, it is not clear if the authorities will conduct random audits of companies doing business in the EU, or if the authorities will wait for complaints to be filed by individuals who claim their rights have been violated. Enforcement uncertainty and the costs associated with ensuring GDPR compliance may be onerous and adversely affect our business, financial condition, results of operations, and prospects.

We may be exposed to potential liability related to the development, testing or manufacturing of compounds we develop and our insurance coverage may not be sufficient to cover losses.

We are developing, clinically testing and manufacturing potential therapeutic products for use in humans. In connection with these activities, we could be liable if persons are injured or die while using these drugs. We may have to pay substantial damages and/or incur legal costs to defend claims resulting from injury or death, and we may not receive expected royalty or milestone payments if commercialization of a drug is limited or ended as a result of such claims. We have product liability and clinical trial insurance that contains customary exclusions and provides coverage per occurrence at levels, in the aggregate, which we believe are customary and commercially reasonable in our industry given our current stage of drug development. Our product liability insurance does not cover every type of product liability claim that we may face or loss we may incur and may not adequately compensate us for the entire amount of covered claims or losses or for the harm to our business reputation. Also, we may be unable to maintain our current insurance policies or obtain and maintain necessary additional coverage at acceptable costs, or at all.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could significantly harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Although we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities that may arise from employee injury. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts, which could adversely affect our business, financial condition, results of operations or prospects. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the Foreign Corrupt Practices Act (FCPA), the U.K. Bribery Act 2010 (Bribery Act), and other anticorruption laws that apply in countries

TABLE OF CONTENTS

where we do business and may do business in the future. The FCPA, the Bribery Act, and these other laws generally prohibit us, our officers and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential FCPA or Bribery Act violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the FCPA, the Bribery Act, or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted.

We are also subject to other laws and regulations, including regulations administered by the governments of the United States, United Kingdom, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, which we collectively refer to as Trade Control Laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the FCPA, the Bribery Act, or other legal requirements, including Trade Control Laws. If we are not in compliance with the FCPA, the Bribery Act, and other anti-corruption laws or Trade Control Laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Likewise, any investigation of any potential violations of the FCPA, the Bribery Act, other anti-corruption laws or Trade Control Laws by U.S., U.K. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

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

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers, and purchasers, subject to various federal and state fraud and abuse laws and regulations. These laws will impact, among other things, our proposed sales, marketing, and educational programs. In addition, we may be subject to patient privacy laws enacted by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to the following:

- Anti-Kickback Statute: prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, or receiving remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchasing, ordering, leasing, arranging for, or recommending the purchasing, ordering, or leasing of, any good or service for which payment may be made, in whole or in part, under a federal health care program such as Medicare or Medicaid;

- False Claims Act: the federal civil and criminal false claims laws, including the civil False Claims Act, and Civil Monetary Penalties Law, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, false or fraudulent claims for payment or knowingly making, using or causing to made or used a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government, or knowingly concealing or knowingly and improperly avoiding or decreasing an obligation to pay money to the federal government.

- HIPAA: HIPAA created additional federal criminal statutes that prohibit, among other things, executing a scheme to defraud any health care benefit program or making false statements relating to health care matters, and apply regardless of the payor (e.g., public or private);

- HIPAA and HITECH: HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, which impose obligations

TABLE OF CONTENTS

on HIPAA covered entities and their business associates, including mandatory contractual terms and required implementation of administrative, physical and technical safeguards to maintain the privacy and security of individually identifiable health information;

•

Transparency Requirements: the federal physician transparency requirements known as the Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or the Affordable Care Act (ACA), and similar states laws, which requires manufacturers of drugs, medical devices, biological and medical supplies covered by Medicare, Medicaid, or State Children's Health Insurance Program (CHIP), with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services information related to payments and other transfers of value made by that entity to physicians and other healthcare providers and their immediate family members and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and

•

Analogous State, Local and Foreign Laws: analogous state, local and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader than similar federal laws, can apply to claims involving health care items or services regardless of payor, including commercial insurers, and are enforced by many different federal and state agencies as well as through private actions.

Efforts to ensure that our business arrangements with third parties will comply with applicable health care laws and regulations will involve substantial costs. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, 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.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of the member states of the European Union, such as the Bribery Act. Infringement of these laws could result in substantial fines and imprisonment.

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

RISKS RELATED TO EMPLOYEES, FACILITIES AND INFORMATION TECHNOLOGY

Our operations could be interrupted by damage to our laboratory facilities.

The efficiency of certain of our operations depends in part upon the continued use of our specialized laboratories and equipment in Bedford, Massachusetts. In the first half of 2019 we plan to transition our laboratory and associated equipment into a new facility in Woburn, Massachusetts. Catastrophic events, including fires or explosions, could damage our laboratories, equipment, scientific data, work in progress or inventories of chemical compounds and biological materials and may materially interrupt that aspect of our business. We employ safety precautions in our laboratory activities in order to reduce the likelihood of the occurrence of these catastrophic events; however, we cannot eliminate the chance that such an event will occur. Rebuilding or relocating our facilities could be time consuming and result in substantial delays in our development of products and in fulfilling our agreements with third parties, including our collaborators.

TABLE OF CONTENTS

Security breaches may disrupt our operations and adversely affect our operating results.

Our network security and data recovery measures and those of third parties with which we contract, may not be adequate to protect against computer viruses, cyber-attacks, break-ins, and similar disruptions from unauthorized tampering with our computer systems. The misappropriation, theft, sabotage or any other type of security breach with respect to any of our proprietary and confidential information that is electronically stored, including research or clinical trial data, could cause interruptions in our operations, and could result in a material disruption of our clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. This disruption could have a material adverse impact on our business, operating results and financial condition. Any such breach, loss, or compromise of clinical trial participant personal data may also subject us to civil fines and penalties, either under the GDPR and relevant member state law in the European Union, and HIPAA and other relevant state and federal privacy laws in the United States. Additionally, any break-in or trespass of our facilities that results in the misappropriation, theft, sabotage or any other type of security breach with respect to our proprietary and confidential information, including research or clinical trial data, or that results in damage to our research and development equipment and assets could have a material adverse impact on our business, operating results, and financial condition.

RISKS RELATED TO OUR COMMON STOCK

Our stock price may be extremely volatile.

The trading price of our common stock has been highly volatile. We believe the trading price of our common stock will remain highly volatile and may fluctuate substantially due to factors such as:

- results or timing of clinical trials;
- announcement of FDA approval or non-approval, or delays in the FDA review process, of our or our collaborators' product candidates or those of our competitors or actions taken by regulatory agencies with respect to our, our collaborators' or our competitors' clinical trials;
- announcement of new products by us or our competitors;
- quarterly variations in our or our competitors' results of operations, including as a result of recognition of upfront licensing or other fees, the timing and amount of expenses incurred for clinical development, regulatory approval and commercialization of our product candidates;
- litigation, including intellectual property infringement lawsuits, involving us;
- financing transactions;
- developments in the biotechnology and pharmaceutical industries;
- the general performance of the equity markets and in particular the biopharmaceutical sector of the equity markets;
- departures of key personnel or board members;

- developments concerning current or future collaborations;

- FDA or international regulatory actions affecting our industry generally; and

- third-party reimbursement policies.

This volatility and general market declines in our industry over the past several years have affected the market prices of securities issued by many companies, often for reasons unrelated to their operating performance, and may adversely affect the price of our common stock. In the past, securities class action litigation has often been instituted following periods of volatility in the market price of a company's securities. A securities class action suit against us could result in potential liabilities, substantial costs and the diversion of management's attention and resources, regardless of the outcome of the action.

TABLE OF CONTENTS

Some of our existing stockholders can exert control over us, and their interests could conflict with the best interests of our other stockholders.

Due to their combined stock holdings, our principal stockholders (stockholders holding more than 5% of our common stock), acting together, may be able to exert significant influence over all matters requiring stockholder approval, including the election of directors and approval of significant corporate transactions. In addition, this concentration of ownership may delay or prevent a change in control of our company, even when a change may be in the best interests of our stockholders. Furthermore, the interests of these stockholders may not always coincide with our interests as a company or the interests of other stockholders. Accordingly, these stockholders could cause us to enter into transactions or agreements that would not be widely viewed as beneficial.

If our officers, directors or principal stockholders sell substantial amounts of our common stock (including shares issued upon the exercise of options) in the public market, the market price of our common stock could fall. These sales also might make it more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate.

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 or deter attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and bylaws and Delaware law may discourage, delay or prevent an acquisition of our company, a change in control, or attempts by our stockholders to replace or remove members of our current 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 Board of Directors having three classes of directors with a three-year term of office that expires as to one class each year, commonly referred to as a “staggered board”;

- a prohibition on actions by our stockholders by written consent;

- the inability of our stockholders to call special meetings of stockholders;

- the ability of our Board of Directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our Board of Directors;

- limitations on the removal of directors; and

- advance notice requirements for director nominations and stockholder proposals.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. As a result, it is difficult for a third party to acquire control of us without the approval of our Board of Directors and, therefore, mergers with and acquisitions of us that our stockholders may consider in their best interests may not occur.

Because we do not intend to pay dividends, stockholders will benefit from an investment in our common stock only if it appreciates in value.

We have never declared or paid any cash dividends on our common stock. We currently intend to retain our future earnings, if any, to finance the expansion of our business and do not expect to pay any cash dividends in the foreseeable future. As a result, the success of an investment in our common stock will depend entirely upon any future appreciation. There is no guarantee that our common stock will appreciate in value or even maintain the price at which stockholders have purchased their shares.

TABLE OF CONTENTS

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

In January 2015, we entered into a lease agreement for our headquarters facility of approximately 15,000 square feet. The lease commenced on May 1, 2015 for a term of five years and three months with an average annual base rent of $455 thousand. See Note 5, “Property and Equipment” in the Notes to Financial Statements appearing in Item 8 in this Annual Report on Form 10-K.

ITEM 3. LEGAL PROCEEDINGS

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

TABLE OF CONTENTS

PART II

ITEM 5.

MARKET FOR THE REGISTRANT'S COMMON STOCK, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

STOCK PERFORMANCE GRAPH

The following graph shows the cumulative total stockholder return on our common stock over the period from December 31, 2013 to December 31, 2018, as compared with that of the NASDAQ Stock Market Index (U.S. Companies) and the NASDAQ Biotechnology Index, based on an initial investment of $100 in each on December 31, 2013. Total stockholder return is measured by dividing share price change plus dividends, if any, for each period by the share price at the beginning of the respective period, and assumes reinvestment of dividends.

COMPARISON OF CUMULATIVE TOTAL RETURN OF ARQULE, INC.,
NASDAQ STOCK MARKET (U.S. COMPANIES) INDEX
AND NASDAQ BIOTECHNOLOGY INDEX

	12/31/13	12/31/14	12/31/15	12/31/16	12/31/17	12/31/18
ArQule, Inc.	100.00	56.74	100.93	58.60	76.74	128.84
NASDAQ Market (U.S. Companies) Index	100.00	112.46	113.00	127.70	155.01	146.57
NASDAQ Biotechnology Index	100.00	134.40	150.22	118.15	143.71	130.97

ArQule's common stock is traded on the NASDAQ Global Market under the symbol "ARQL". As of February 15, 2019, there were approximately 74 holders of record and approximately 9,893 beneficial stockholders of our common stock.

EQUITY COMPENSATION PLAN INFORMATION

(Amounts in thousands except per share amounts)

Plan Category	Number of Shares of Common Stock to be Issued Upon Exercise of Outstanding Options	Weighted-Average Exercise Price of Outstanding Options	Weighted-Average Remaining Contractual Term (in years)	Number of Shares of Common Stock Remaining Available for Future Issuance Under Equity Compensation Plans
Equity compensation plans approved by stockholders	10,748,157	$ 2.90	6.08	4,610,616
Equity compensation plans not approved by stockholders	—	—	—	—

TABLE OF CONTENTS

ITEM 6. SELECTED FINANCIAL DATA

The following selected financial data have been derived from our audited historical financial statements, certain of which are included elsewhere in this Annual Report on Form 10-K. The following selected financial data should be read in conjunction with our financial statements and related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" appearing elsewhere in this Annual Report on Form 10-K.

The following data is in thousands, except per share data.

	YEAR ENDED DECEMBER 31,				
	2018	2017	2016	2015	2014
STATEMENT OF OPERATIONS AND COMPREHENSIVE LOSS DATA:					
Revenue:					
Research and development revenue(a)	$ 25,764	$ —	$ 4,709	$ 11,239	$ 11,254
Costs and expenses:					
Research and development(b)	28,710	19,468	20,042	15,561	22,271
General and administrative	10,753	7,551	7,563	9,830	12,154
Restructuring and other costs(c)	—	—	—	—	1,099
Total costs and expenses	39,463	27,019	27,605	25,391	35,524
Loss from operations	(13,699)	(27,019)	(22,896)	(14,152)	(24,270)
Interest income	1,435	238	178	101	272
Interest expense	(1,666)	(1,520)	—	—	(35)
Other income (expense)(d)	(1,552)	(902)	—	277	642
Loss before income taxes	(15,482)	(29,203)	(22,718)	(13,774)	$ (23,391)
Provision for income taxes	—	—	—	—	—
Net loss	(15,482)	(29,203)	(22,718)	(13,774)	(23,391)
Unrealized gain (loss) on marketable securities	(79)	(18)	(1)	13	(77)
Comprehensive loss	$ (15,561)	$ (29,221)	$ (22,719)	$ (13,761)	$ (23,468)
Basic and diluted net loss per share	$ (0.16)	$ (0.39)	$ (0.33)	$ (0.22)	$ (0.37)
Weighted average common shares outstanding—basic and diluted	99,035	74,813	69,714	62,808	62,627
Cash, cash equivalents and marketable securities(e)	$ 99,558	$ 48,036	$ 31,126	$ 38,772	$ 59,208
Marketable securities-long term	—	—	—	—	2,058
	$ 99,558	$ 48,036	$ 31,126	$ 38,772	$ 61,266
Working capital(e)	$ 91,788	$ 38,824	$ 23,248	$ 28,661	$ 42,824
Notes payable	14,760	14,607	—	—	—
Total assets	106,676	48,902	32,380	40,004	63,394
Total stockholders' equity(e)	78,968	14,181	23,680	29,179	40,545

(a)

Revenue increased in 2018 compared to 2017 due to revenue of $5.9 million from our February 2018 Sinovant licensing agreement, $18.5 million from our April 2018 Basilea licensing agreement and $1.3 million from a

non-exclusive license agreement for certain of our library compounds.

Revenue decreased in 2017 compared to 2016 due to revenue decreases of $2.8 million from our development agreement with Daiichi Sankyo Co., Ltd. (Daiichi Sankyo) and $1.9 million from our license agreement with Kyowa Hakko Kirin Co., Ltd. (Kyowa Hakko Kirin) upon the completion of the development period for both programs on December 31, 2016. In 2017, we announced that the trials of tivantinib did not meet their primary end points and as a result, we, Daiichi Sankyo and Kyowa Hakko Kirin have all discontinued development of tivantinib.

TABLE OF CONTENTS

Revenue decreased in 2016 compared to 2015 due to revenue decreases of $2.7 million from our Daiichi Sankyo development agreement and $3.8 million from our Kyowa Hakko Kirin license agreement.

(b)

The $9.2 million increase in research and development expense in 2018 compared to 2017 was primarily due to higher outsourced preclinical, clinical and product development costs of $8.5 million and $0.7 million from labor and related costs.

The $4.5 million increase in research and development expense in 2016 compared to 2015 was primarily due to increased outsourced clinical and product development costs of $5.3 million, and professional fees of $0.1 million, partially offset by lower labor related costs of $0.5 million and facility costs of $0.4 million.

The $6.7 million decrease in research and development expense in 2015 compared to 2014 was primarily due to lower labor related costs of $2.4 million from reduced headcount, outsourced clinical and product development costs of $1.7 million, facility costs of $1.9 million and lab expenses of $0.7 million.

(c)

On July 30, 2014, we approved plans to restructure our operations to better align our human and financial resources with our primary focus on clinical stage development programs and to extend our cash runway beyond the anticipated time for achievement of key milestones. Commencing on August 4, 2014, we began to reduce our workforce from 62 to approximately 40 employees by the end of the year. Most of this reduction came from our Discovery Group, which has been engaged primarily in early-stage, pre-clinical research. The costs associated with this action were comprised of severance payments of $662 thousand and benefits continuation costs of $74 thousand. In the year ended December 31, 2014, $319 thousand of these costs was paid and the remaining amount was paid in 2015. In addition, in the year ended December 31, 2014, we incurred non-cash charges of $83 thousand related to the modification of employee stock options, and $280 thousand for impairment of property and equipment impacted by the restructuring.

(d)

Other income (expense) in 2018 includes a non-cash expense of $1,552 thousand from the increase in fair value of our preferred stock warrant liability. Other income (expense) in 2017 includes a non-cash expense of $902 thousand from the increase in fair value of our preferred stock warrant liability. Other income (expense) in 2015 includes a gain of $277 thousand from the sale of property and equipment. Other income (expense) in 2014 includes a gain of $254 thousand upon the redemption of $2.1 million of auction rate securities at face value, and a gain of $388 thousand from the sale of property and equipment.

(e)

In July 2018, we sold 12,650,000 shares of common stock at $5.50 per share for aggregate net proceeds of approximately $64.6 million after commissions and other estimated offering expenses.

In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering we raised net proceeds of $9.5 million through the sale of 8,370 shares of series A convertible preferred stock (Series A Preferred) and warrants covering 2,259 shares of Series A Preferred (Warrants). Each share of Series A Preferred converted into 1,000 shares of common stock and each associated Warrant converted into 1,000 common stock warrants upon the effectiveness on May 8, 2018 of an amendment to our restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The Warrants have a post-conversion exercise price of $1.75 per share, are exercisable immediately and expire in May 2022.

In October 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering, we issued 13,938,651 shares of our common stock and warrants for 3,123,674 shares of our common stock for aggregate net proceeds of $15.6 million. Each warrant is exercisable for $1.75 per share and expires in October 2021.

In September 2017, we sold 2.0 million shares of common stock through an at-the-market (ATM) offering and raised net proceeds of approximately $2.3 million.

TABLE OF CONTENTS

In February 2016, we entered into definitive stock purchase agreements with certain institutional and accredited investors. In conjunction with this stock offering we issued 8,027,900 shares of our common stock and non-transferable options for 3,567,956 shares of our common stock for aggregate net proceeds of $15.2 million. Each option was exercisable for $2.50 per share and they all expired in March 2017.

TABLE OF CONTENTS

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

The following discussion should be read in conjunction with our financial statements and related notes contained in this report.

We are a biopharmaceutical company engaged in the research and development of innovative therapeutics to treat cancers and rare diseases. Our mission is to discover, develop and commercialize novel small molecule drugs in areas of high unmet need that will dramatically extend and improve the lives of our patients. These product candidates target biological pathways implicated in a wide range of cancers and certain non-oncology indications. Our discovery and development efforts are guided, when possible, by an understanding of the role of biomarkers, which are indicators of a particular biological condition or process and may predict the clinical benefit of our compounds in defined patient populations. Our clinical-stage pipeline consists of four product candidates, all of which are in targeted patient populations, making ArQule a leader among companies our size in precision medicine.

ArQule has a long history of kinase drug discovery and development, having discovered and introduced ten kinase inhibitors into clinical trials. Our drug discovery efforts have been informed by our historical expertise in chemistry, our work in rational drug design and by our insight into kinase binding and regulation. We have applied this knowledge to produce significant chemical matter for a number of kinase targets and to build an extensive library of proprietary compounds with the potential to target multiple kinases in oncology and other therapeutic areas, such as rare diseases. We may bring further preclinical programs forward and interrogate our library against new targets beyond kinases either directly or with collaborators.

Our pipeline of orally bioavailable product candidates is directed toward molecular targets and biological processes with demonstrated roles in the development of both human cancers and rare, non-oncology diseases. All of these programs are being developed in targeted, biomarker-defined patient populations. By seeking out subgroups of patients that are most likely to respond to our product candidates, we seek to identify small, often orphan, indications that allow for focused and efficient development. At the same time, in addition to pursuing these potentially fast-to-market strategies, we also pursue development in other indications that could allow us to expand the utility of the product candidates if approved. Our clinical pipeline includes the following product candidates:

- ARQ 531 is a potent and reversible dual inhibitor of both wild type and C481S-mutant Bruton's tyrosine kinase (BTK) that is in Phase 1 clinical development for B-cell malignancies refractory to other therapeutic options

- Miransertib (ARQ 092) is a potent and selective inhibitor of protein kinase B (AKT), a serine/ threonine kinase. We expect to commence a registrational clinical trial of miransertib for the treatment of Proteus syndrome and PIK3CA-Related Overgrowth Syndromes (PROS) in the first half of 2019. Miransertib is also in Phase 1b clinical development in oncology in combination with the hormonal therapy, anastrozole, in endometrial cancer

- ARQ 751 is a next-generation, highly potent and selective inhibitor of AKT that is in Phase 1 clinical development for solid tumors harboring AKT, phosphoinositide 3-kinase (PI3K) or phosphatase and tensin homolog (PTEN) loss mutations

- Derazantinib (ARQ 087) is a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family of kinases that is in a registrational clinical trial in intrahepatic cholangiocarcinoma (iCCA) in patients with FGFR2 fusions. Derazantinib was exclusively licensed to Basilea Pharmaceutica Limited (Basilea) in April 2018 in the United States, European Union, Japan and the rest of the world, excluding the People's Republic of China, Hong Kong, Macau, and Taiwan (collectively, Greater China) where derazantinib was exclusively licensed to Sinovant Sciences Ltd., a subsidiary of Roivant Sciences Ltd. (Sinovant) in February 2018

We have incurred a cumulative deficit of approximately $548 million from inception through December 31, 2018. We recorded a net loss for 2016, 2017 and 2018 and expect a net loss for 2019.

TABLE OF CONTENTS

LIQUIDITY AND CAPITAL RESOURCES

	December 31,			% increase (decrease)	
	2018	2017	2016	2017 to 2018	2016 to 2017
	(in millions)				
Cash, cash equivalents and marketable securities short-term	$ 99.6	$ 48.0	$ 31.1	107%	54%
Working capital	91.8	38.8	23.2	136%	67%

	Year Ended December 31,		
	2018	2017	2016
	(in millions)		
Cash flow from:			
Operating activities	$ (13.0)	$ (25.2)	$ (22.9)
Investing activities	(53.3)	(11.9)	8.9
Financing activities	65.3	42.1	15.4

Cash flow from operating activities. Our uses of cash for operating activities have primarily consisted of salaries and wages for our employees, facility and facility-related costs for our offices and laboratories, fees paid in connection with preclinical and clinical studies, laboratory supplies and materials and professional fees. The sources of our cash flow from operating activities have consisted primarily of payments received from our collaborators for services performed or upfront payments for license agreements or future services. In the year ended December 31, 2018, our net use of cash was primarily driven by the difference between cash received from our collaborations and payments for operating expenses which resulted in net cash outflows of $13.0 million. For the years ended December 31, 2017 and 2016 our net use of cash was primarily driven by payments for operating expenses which resulted in net cash outflows of $25.2 million, and $22.9 million, respectively.

Cash flow from investing activities. Our net cash used by investing activities of $53.3 million and $11.9 million in the years ended December 31, 2018 and 2017, respectively was comprised of net purchases of marketable securities. Our net cash provided by investing activities of $8.9 million in 2016 was principally comprised of net maturities of marketable securities. The composition and mix of cash, cash equivalents and marketable securities may change frequently as a result of our constant evaluation of conditions in financial markets, the maturity of specific investments, and our near-term liquidity needs.

Our cash equivalents and marketable securities typically include commercial paper, money market funds, and U.S. Treasury bill funds, which have investment grade ratings. Our cash equivalents and our portfolio of marketable securities are subject to market risk due to changes in interest rates. Fixed rate interest securities may have their market value adversely impacted due to a rise in interest rates, while floating rate securities may produce less income than expected if interest rates fall. Due in part to these factors, our future investment income may fall short of expectation due to changes in interest rates or we may suffer losses in principal if we are forced to sell securities that decline in market value due to changes in interest rates.

Cash flow from financing activities. Our net cash provided by financing activities of $65.3 million in year ended December 31, 2018 was principally comprised of net proceeds from our July 2018 stock offering of $64.6 million and $0.7 million from stock option exercises. Our net cash provided by financing activities of $42.1 million in year ended December 31, 2017 was principally comprised of net proceeds of $14.6 million from the loan and security agreement (the “Loan Agreement”) that we entered into in January 2017, and net proceeds from our 2017 stock offerings of $27.4 million. Our net cash provided by financing activities of $15.4 million in the year ended December 31, 2016, was comprised of net proceeds from our February 2016 stock offering of $15.2 million and $0.2 million from stock option exercises and employee stock plan purchases.

Our cash requirements may vary materially from those now planned depending on the results of our drug discovery and development strategies, our ability to enter into additional corporate collaborations and the terms of such

collaborations, results of research and development, unanticipated required capital

TABLE OF CONTENTS

expenditures, competitive and technological advances, acquisitions and other factors. We cannot guarantee that we will be able to develop any of our product candidates into a commercial product.

In January 2017, we entered into a loan and security agreement with Oxford Finance, LLC with a principal balance of $15 million (see Note 8). The terms of the agreement, as amended in February 2018, requires payments of interest on a monthly basis through August 2019 and payments of interest and principal from September 2019 to August 2022.

In February 2016, we entered into definitive stock purchase agreements with certain institutional and accredited investors. In conjunction with this stock offering, we issued 8,027,900 shares of our common stock for aggregate net proceeds of $15.2 million

In September 2017, we sold 2.0 million shares of common stock through an at-the-market (ATM) offering and raised net proceeds of $2.3 million.

In October 2017, we entered into definitive stock purchase agreements with certain institutional investors. In conjunction with this stock offering we issued 13,938,651 shares of our common stock and warrants to purchase 3,123,674 shares of our common stock for aggregate net proceeds of $15.6 million. Each warrant is exercisable at a price of $1.75 per share and expires in October 2021.

In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering, we raised net proceeds of $9.5 million through the sale of 8,370 shares of series A convertible preferred stock (Series A Preferred) and warrants to purchase 2,259 shares of Series A Preferred (Warrants). Each share of Series A Preferred converted into 1,000 shares of common stock and each associated Warrant converted into 1,000 common stock warrants upon the effectiveness on May 8, 2018 of an amendment to our restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The Warrants have a post-conversion exercise price of $1.75 per share, are exercisable immediately and expire in May 2022.

In February 2018, we entered into a License Agreement with Sinovant pursuant to which ArQule granted Sinovant an exclusive license to develop and commercialize derazantinib in Greater China. The agreement provides for an upfront payment to ArQule of $3 million and a $2.5 million development milestone that was paid in the first quarter of 2019. We are also eligible for up to an additional $82 million in regulatory and sales milestones. Upon commercialization, we are entitled to receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in Greater China. Sinovant will be responsible for all costs and expenses of development, manufacture and commercialization in Greater China. For the year ended December 31, 2018 we recognized revenue of $5.9 million related to the Sinovant agreement, including $0.4 million for certain research and development services that we provided.

In April 2018, we entered into a License Agreement with Basilea pursuant to which ArQule granted Basilea an exclusive license to develop and commercialize derazantinib in the United States, European Union, Japan and the rest of the world, excluding Greater China. Under the terms of the agreement, we received an upfront payment of $10 million and are eligible for up to $326 million in regulatory and commercial milestones. Upon commercialization, we are entitled to receive staggered royalties on future net sales of derazantinib ranging from the high-single digits to the mid-teens on direct sales and mid-single digits to low-double digits on indirect sales. Basilea will be responsible for all costs and expenses of development, manufacture and commercialization in its territory. Under certain circumstances, we may have the opportunity to promote derazantinib in the United States directly. Revenue for the year ended December 31, 2018 totaled $18.5 million for providing the technology license as well as certain research and development services to Basilea, recognized as revenue on a cost-to-cost method.

In July 2018, we sold 12,650,000 shares of common stock at $5.50 per share for aggregate net proceeds of approximately $64.6 million after commissions and other offering expenses.

We anticipate that our cash, cash equivalents and marketable securities on hand at December 31, 2018, financial support from our licensing agreements, and the one year extension of our loan agreement mentioned above will be sufficient to finance our operations into 2021 which is in excess of at least 12 months from the issuance date of these financial statements.

TABLE OF CONTENTS

We expect that we will need to raise additional capital or incur indebtedness to continue to fund our operations in the future. Our ability to raise additional funds will depend on financial, economic and market conditions, and due to global capital and credit market conditions or for other reasons, we may be unable to raise capital when needed, or on terms favorable to us. If necessary funds are not available, we may have to delay, reduce the scope of, or eliminate some of our development programs, potentially delaying the time to market for any of our product candidates.

Our contractual obligations were comprised of the following as of December 31, 2018 (in thousands):

	Payment due by period				
Contractual Obligations	Total	Less than 1 year	1–3 years	3–5 years	More than 5 years
Notes payable	$ 15,900	$ 1,667	$ 10,000	$ 4,233	$ —
Interest on notes payable	3,461	1,143	1,322	996	—
Operating lease obligations	819	523	296	—	—
Purchase obligations	8,497	8,497	—	—	—
Total	$ 28,677	$ 11,830	$ 11,618	$ 5,229	$ —

In January 2015, we entered into a lease agreement for our headquarters facility. The lease commenced on May 1, 2015 for a term of five years and three months with an average annual base rent of $455 thousand. The obligations for this facility are included in the table above.

The notes payable relate to the loan and security agreement we entered into with Oxford Finance, LLC in January 2017. The maturity date of the notes payable is August 1, 2022, with repayment of principal commencing on September 1, 2019.

Purchase obligations are comprised primarily of non-cancelable outsourced preclinical and clinical trial expenses, product development and other costs to support our ongoing research and development efforts.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

A “critical accounting policy” is one which is both important to the portrayal of our financial condition and results and requires management’s most difficult, subjective or complex judgments, often as a result of the need to make estimates about the effect of matters that are inherently uncertain. Management believes the following are critical accounting policies. For additional information, please see the discussion of our significant accounting policies in Note 2 to the Financial Statements included in Item 8 of this Form 10-K.

Research and Development Revenue

Research and development revenue is generated primarily through collaborative research and development agreements. The terms of the agreements may include nonrefundable upfront payments, funding for research and development, milestone payments and royalties on any product sales derived from collaborations.

Our policy is to recognize revenues when our customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. We recognize revenues following the five step model prescribed under Accounting Standards Codification Topic 606—Revenue from Contracts with Customers. Under Topic 606 we: (i) identify contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenues when (or as) we satisfy the performance obligation. We only apply the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, we assess the goods or services promised within each contract, and determine those that are performance obligations. The transaction price is allocated to the identified performance obligations in proportion to their estimated standalone selling prices (SSP) on a relative basis. Determining the estimated SSP for performance

TABLE OF CONTENTS

obligations requires judgment. In developing the SSP for a performance obligation, we consider applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. Revenue is recognized when each distinct performance obligation is satisfied. For certain performance obligations that are satisfied over time, we recognize revenue using the cost-to-cost method. Under the cost-to-cost method, the extent of progress towards completion is measured based on the ratio of actual costs incurred to the total estimated costs expected upon satisfying the identified performance obligation. Under this method, revenue will be recorded as a percentage of the estimated transaction price based on the extent of progress towards completion. Incurred cost represents work performed, which corresponds with, and thereby best depicts, the transfer of control to the customer. Due to the nature of the work performed in these arrangements, the estimation of cost at completion is subject to many variables and requires judgment. Circumstances can arise that change original estimates of costs or progress toward completion. Changes to the estimated cost at completion have not had a material impact on the results of operations.

Stock-Based Compensation

Our stock-based compensation cost is measured at the grant date, based on the calculated fair value of the award, and is recognized as an expense over the employees' requisite service period (generally the vesting period of the equity grant). We estimate the fair value of stock options using the Black-Scholes valuation model. Key input assumptions used to estimate the fair value of stock options include the exercise price of the award, expected option term, expected volatility of our stock over the option's expected term, risk-free interest rate over the option's expected term, and the expected annual dividend yield. We believe that the valuation technique and approach utilized to develop the underlying assumptions are appropriate in calculating the fair values of our stock option grants.

Cash Equivalents and Marketable Securities

We generally classify our marketable securities as available-for-sale at the time of purchase and re-evaluate such designation as of each balance sheet date. We classify our investments as either current or long-term based upon the investments' contractual maturities and our ability and intent to convert such instruments to cash within one year. We report available-for-sale investments at fair value as of each balance sheet date and include any unrealized gains and, to the extent deemed temporary, unrealized losses in stockholders' equity. Realized gains and losses are determined using the specific identification method and are included in other income in the statement of operations and comprehensive loss.

We conduct quarterly reviews to determine the fair value of our investment portfolio and to identify and evaluate each investment that has an unrealized loss, in accordance with the meaning of other-than-temporary impairment and its application to certain investments. An unrealized loss exists when the current fair value of an individual security is less than its amortized cost basis. In the event that the cost basis of a security exceeds its fair value, we evaluate, among other factors, the duration of the period that, and extent to which, the fair value is less than cost basis, the financial health of and business outlook for the issuer, including industry and sector performance, and operational and financing cash flow factors, overall market conditions and trends, our intent to sell the investment and if it is more likely than not that we would be required to sell the investment before its anticipated recovery. Unrealized losses on available-for-sale securities that are determined to be temporary, and not related to credit loss, are recorded in accumulated other comprehensive income loss.

For available-for-sale debt securities with unrealized losses, we perform an analysis to assess whether we intend to sell or whether we would more likely than not be required to sell the security before the expected recovery of the amortized cost basis. Where we intend to sell a security, or may be required to do so, the security's decline in fair value is deemed to be other-than-temporary and the full amount of the unrealized loss is reflected in the statement of operations and comprehensive loss as an impairment loss.

Regardless of our intent to sell a security, we perform additional analysis on all securities with unrealized losses to evaluate losses associated with the creditworthiness of the security. Credit losses are identified where we do not expect to receive cash flows sufficient to recover the amortized cost basis of a security.

TABLE OF CONTENTS

RESULTS OF OPERATIONS

The following are the results of operations for the years ended December 31, 2018, 2017 and 2016:

Revenue

				% increase (decrease)	
	2018	2017	2016	2017 to 2018	2016 to 2017
	(in millions)				
Research and development revenue	$ 25.8	$ —	$ 4.7	100%	(100)%

2018 as compared to 2017: Research and development revenue in 2018 consisted of $5.9 million from our February 2018 Sinovant licensing agreement, $18.5 million from our April 2018 Basilea licensing agreement and $1.3 million from a non-exclusive license agreement for certain of our library compounds. We did not have any research and development revenue in 2017.

2017 as compared to 2016: We did not have any research and development revenue in 2017. In 2016, research and development revenue consisted of $2.8 million from our Daiichi Sankyo development agreement and $1.9 million from our Kyowa Hakko Kirin license agreement. We are no longer developing product candidates under the Daiichi Sankyo agreement or the Kyowa Hakko Kirin agreement and do not expect to receive revenue from either agreement in future periods.

Research and development

				% increase (decrease)	
	2018	2017	2016	2017 to 2018	2016 to 2017
	(in millions)				
Research and development	$ 28.7	$ 19.5	$ 20.1	47%	(3)%

2018 as compared to 2017: – The $9.2 million increase in research and development expense in 2018 was primarily due to higher outsourced preclinical, clinical and product development costs of $8.5 million and $0.7 million from labor and related costs. At December 31, 2018 we had 21 employees dedicated to our research and development program, up from 18 employees at December 31, 2017.

2017 as compared to 2016: The $0.6 million decrease in research and development expense in 2017 was primarily due to lower labor related costs of $0.6 million and professional fees of $0.2 million, partially offset by higher outsourced clinical and product development costs for our pipeline programs of $0.3 million. At December 31, 2017 we had 18 employees dedicated to our research and development program, down from 21 employees at December 31, 2016.

Overview

Our research and development expense consists primarily of salaries and related expenses for personnel, costs of contract manufacturing services, costs of facilities and equipment, fees paid to professional service providers in conjunction with our clinical trials, fees paid to research organizations in conjunction with preclinical animal studies, costs of materials used in research and development, consulting, license, and sponsored research fees paid to third parties and depreciation of associated laboratory equipment. We expect our research and development expenses to remain significant, yet consistent, as we continue to develop our portfolio of oncology and rare disease programs.

We have not accumulated and tracked our internal historical research and development costs or our personnel and personnel-related costs on a program-by-program basis. Our employee and infrastructure resources are allocated across several projects, and many of our costs are directed to broadly applicable research endeavors. As a result, we cannot state the costs incurred for each of our programs on a program-by-program basis. Our future research and development expenses in support of our current and future oncology programs will be subject to numerous uncertainties in timing and cost to completion. We test potential products in numerous preclinical studies for safety, toxicology, and efficacy. We may conduct multiple clinical trials for each product. As we obtain results from clinical trials, we may elect to discontinue

TABLE OF CONTENTS

or delay clinical trials for certain products in order to focus our resources on more promising products. Completion of clinical trials may take several years or more, and the length of time and cost of development generally varies substantially according to the type, complexity, novelty, and intended use of a product.

We estimate that clinical trials of the type generally needed to secure new drug approval are typically completed over the following timelines:

Clinical Phase	Estimated Completion Period
Phase 1	1–2 years
Phase 2	2–3 years
Phase 3	2–4 years

The duration and the cost of clinical trials may vary significantly over the life of a project as a result of differences arising during clinical development, including, among others, the following:

- the number of clinical sites included in the trials;
- the length of time required to enroll suitable patients;
- the number of patients that ultimately participate in the trials;
- the duration of patient follow-up to ensure the absence of long-term product-related adverse events; and
- the efficacy and safety profile of the product.

An element of our business strategy is to pursue the research and development of a broad pipeline of products. This is intended to allow us to diversify the risks associated with our research and development expenditures. As a result, we believe our future capital requirements and future financial success do not substantially depend on any one product. To the extent we are unable to build and maintain a broad pipeline of products, our dependence on the success of one or a few products increases.

Our strategy includes entering into alliance arrangements with third parties to participate in the development and commercialization of our products, such as our collaboration agreements with Sinovant and Basilea. In the event that third parties have control over the clinical trial process for a product, the estimated completion date would be under control of that third party rather than under our control. We cannot forecast with any degree of certainty whether our products will be subject to future collaborative arrangements or how such arrangements would affect our development plans or capital requirements.

As a result of the uncertainties discussed above, we make significant estimates in determining the duration and completion costs of our programs or when and to what extent we will receive cash inflows from the commercialization and sale of a product. Our inability to complete our programs in a timely manner or our failure to enter into appropriate collaborative agreements could significantly increase our capital requirements and could adversely impact our liquidity. These uncertainties could force us to seek additional, external sources of financing from time-to-time in order to continue with our product development strategy. Our inability to raise additional capital, or to do so on terms reasonably acceptable to us, would jeopardize the future success of our business.

General and administrative

			% increase (decrease)	
2018	2017	2016		

				2017 to 2018	2016 to 2017
	(in millions)				
General and administrative	$ 10.8	$ 7.6	$ 7.6	42%	—%

2018 compared to 2017: General and administrative expense increased in 2018 by $3.2 million principally due to higher consulting and professional fees of $2.1 million and labor and related costs of $1.1 million. General and administrative headcount was 15 at December 31, 2018 compared to 14 at December 31, 2017.

TABLE OF CONTENTS

2017 compared to 2016: General and administrative expense was constant in 2017 compared with 2016. General and administrative headcount was 14 at December 31, 2017 and 2016.

Interest income, interest expense and other income (expense)

				% increase (decrease)	
	2018	2017	2016	2017 to 2018	2016 to 2017
	(in thousands)				
Interest income	$ 1,435	$ 238	$ 178	503%	34%
Interest expense	(1,666)	(1,520)	—	10%	100%
Other income (expense)	(1,552)	(902)	—	72%	100%

Interest income is comprised of interest income derived from our portfolio of cash, cash equivalents and investments. Interest income increased in 2018 primarily due to an increase in our portfolio balance resulting from (i) net proceeds of $64.6 million from our July 2018 stock offering, (ii) up-front and other payments from our 2018 licensing agreements and (iii) increased interest rates. Interest income increased in 2017 primarily due to an increase in our portfolio balance resulting from net proceeds of $14.6 million from our January 2017 loan and security agreement, and $27.4 million from our 2017 stock offerings.

Interest expense relates to the loan agreement we entered into in January 2017.

Other income (expense) in 2018 and 2017 includes a non-cash expense from the net increase in fair value of our preferred stock warrant liability of $1.6 million and $0.9 million, respectively.

Provision for income taxes

There was no current or deferred tax expense for the years ended December 31, 2018, 2017 or 2016 due to our loss before income taxes and our valuation allowance. We have recorded a full valuation allowance against our deferred tax assets based upon the weight of available evidence, as it is more likely than not that the deferred tax assets will not be realized.

RECENT ACCOUNTING PRONOUNCEMENTS

For a discussion of new accounting pronouncements please read Note 2, Summary of Significant Accounting Policies to our financial statements included in this report.

TABLE OF CONTENTS

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

We own financial instruments that are sensitive to market risk as part of our investment portfolio. We have implemented policies regarding the amount and credit ratings of investments. Our investment portfolio is used to preserve our capital until it is used to fund operations, including our research and development activities. Our investments are evaluated quarterly to determine the fair value of the portfolio.

Our cash equivalents and marketable securities typically include commercial paper, money market funds, and U.S. Treasury bill funds that have investment grade ratings.

Our cash equivalents and our portfolio of marketable securities are subject to market risk due to changes in interest rates. Fixed rate interest securities may have their market value adversely impacted due to a rise in interest rates, while floating rate securities may produce less income than expected if interest rates fall. Due in part to these factors, our future investment income may fall short of expectation due to changes in interest rates or we may suffer losses in principal if we are forced to sell securities that decline in market value due to changes in interest rates. Based on the type of securities we hold, we do not believe a change in interest rates would have a material impact on our financial statements. If interest rates were to increase or decrease by 1%, this would not result in a material change in the fair value of our investment portfolio.

TABLE OF CONTENTS

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

INDEX TO FINANCIAL STATEMENTS

TABLE OF CONTENTS

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of ArQule, Inc.

Opinions on the Financial Statements and Internal Control over Financial Reporting

We have audited the accompanying balance sheets of ArQule, Inc. (the "Company") as of December 31, 2018 and 2017 and the related statements of operations and comprehensive loss, statements of preferred stock and stockholders' equity, and statements of cash flows for each of the three years in the period ended December 31, 2018, including the related notes (collectively referred to as the "financial statements"). We also have audited the Company's internal control over financial reporting as of December 31, 2018, based on criteria established in Internal Control—Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

In our opinion, the financial statements referred to above 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. Also in our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2018, based on criteria established in Internal Control—Integrated Framework (2013) issued by the COSO.

Change in Accounting Principle

As discussed in Note 2 to the financial statements, the Company changed the manner in which it accounts for revenue in 2018.

Basis for Opinions

The Company's management is responsible for these financial statements, for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting, included in Management's Report on Internal Control Over Financial Reporting appearing under Item 9A. Our responsibility is to express opinions on the Company's financial statements and on the Company's internal control over financial reporting 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 audits to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud, and whether effective internal control over financial reporting was maintained in all material respects.

Our audits of the financial statements 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. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

Definition and Limitations of Internal Control over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal

TABLE OF CONTENTS

control over financial reporting includes those policies and procedures that (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts

March 7, 2019

We have served as the Company's auditor since 1994.

TABLE OF CONTENTS

ARQULE, INC.
BALANCE SHEETS

	December 31,	
	2018	2017
	(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)	
ASSETS		
Current assets:		
Cash and cash equivalents	$ 19,236	$ 20,229
Marketable securities-short term	80,322	27,807
Contract receivables	5,984	—
Prepaid expenses	861	547
Total current assets	106,403	48,583
Property and equipment, net	69	115
Other assets	204	204
Total assets	$ 106,676	$ 48,902
LIABILITIES, PREFERRED STOCK AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued expenses	$ 12,948	$ 8,259
Notes payable-current portion	1,667	—
Deferred revenue	—	1,500
Total current liabilities	14,615	9,759
Long-term liabilities:		
Notes payable-long term	13,093	14,607
Warrant liability	—	1,512
Total liabilities	27,708	25,878
Commitments and contingencies (Note 13)		
Preferred stock, convertible, Series A $0.01 par value; 1,000,000, shares authorized; zero and 8,370 shares issued and outstanding at December 31, 2018 and 2017, respectively	—	8,843
Stockholders' equity:		
Common stock, $0.01 par value; 200,000,000 shares authorized; 109,003,637 and 87,110,202 shares issued and outstanding at December 31, 2018 and 2017, respectively	1,090	871
Additional paid-in capital	625,993	547,364
Accumulated other comprehensive loss	(95)	(16)
Accumulated deficit	(548,020)	(534,038)
Total stockholders' equity	78,968	14,181
Total liabilities, preferred stock and stockholders' equity	$ 106,676	$ 48,902

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

59

TABLE OF CONTENTS

ARQULE, INC.
STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
	(IN THOUSANDS, EXCEPT PER SHARE DATA)		
Revenue:			
Research and development revenue	$ 25,764	$ —	$ 4,709
Costs and expenses:			
Research and development	28,710	19,468	20,042
General and administrative	10,753	7,551	7,563
	39,463	27,019	27,605
Loss from operations	(13,699)	(27,019)	(22,896)
Interest income	1,435	238	178
Interest expense	(1,666)	(1,520)	—
Other expense	(1,552)	(902)	—
Loss before income taxes	(15,482)	(29,203)	(22,718)
Provision for income taxes	—	—	—
Net loss	(15,482)	(29,203)	(22,718)
Unrealized loss on marketable securities	(79)	(18)	(1)
Comprehensive loss	$ (15,561)	$ (29,221)	$ (22,719)
Basic and diluted net loss per common share:			
Net loss per common share	$ (0.16)	$ (0.39)	$ (0.33)
Weighted average basic and diluted common shares outstanding	99,035	74,813	69,714

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

TABLE OF CONTENTS

ARQULE, INC.
STATEMENTS OF PREFERRED STOCK AND STOCKHOLDERS' EQUITY
(IN THOUSANDS, EXCEPT SHARE DATA)

	PREFERRED STOCK		COMMON STOCK		ADDITIONAL PAID-IN CAPITAL	ACCUMULATED OTHER COMPREHENSIVE INCOME/(LOSS)	ACCUMULATED DEFICIT	TOTAL STOCK HOLDE EQUIT
	SHARES	AMOUNT	SHARES	PAR VALUE				
Balance at December 31, 2015	—	$ —	62,939,780	$ 629	$ 510,664	$ 3	$ (482,117)	$ 29,1
Issuance of common stock and options from stock offering, net			8,027,900	80	15,094			15,1
Stock option exercises and issuance of common stock			97,498	1	112			113
Restricted shares issued net of forfeitures and shares redeemed for taxes			29,828	—				—
Employee stock purchase plan			51,203	1	67			68
Stock based compensation expense					1,865			1,86
Change in unrealized loss on marketable securities						(1)		(1)
Net loss							(22,718)	(22,7
Balance at December 31, 2016	—	—	71,146,209	$ 711	$ 527,802	2	$ (504,835)	$ 23,6
Issuance of preferred stock and preferred warrants from stock	8,370	8,843						

offerings, net								
Issuance of common stock and common warrants from stock offering, net			15,938,651	160	17,764			17,9
Restricted shares issued net of forfeitures and shares redeemed for taxes			5,380	—				—
Employee stock purchase plan			19,962	—	17			17
Stock based compensation expense					1,434			1,43
Issuance of warrants from notes payable					347			347
Change in unrealized loss on marketable securities						(18)		(18)
Net loss							(29,203)	(29,2
Balance at December 31, 2017	8,370	$ 8,843	87,110,202	$ 871	$ 547,364	$ (16)	$ (534,038)	$ 14,1
Issuance of preferred stock and warrants due to conversion of preferred stock to common stock and preferred warrants to common warrants	(8,370)	(8,843)	8,370,000	83	11,823			11,9
Issuance of common stock			12,650,000	127	64,435			64,5

from stock offering, net								
Stock option exercises and issuance of common stock			603,851	6	750			756
Shares issued from exercise of warrants			269,584	3	(2)			1
Stock based compensation expense					1,503			1,50
Warrants issued upon debt extension					120			120
Change in unrealized loss on marketable securities						(79)		(79)
Increase to opening accumulated deficit upon adoption of new accounting standard							1,500	1,50
Net loss							(15,482)	(15,4
Balance at December 31, 2018	—	$ —	109,003,637	$ 1,090	$ 625,993	$ (95)	$ (548,020)	$ 78,9

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

TABLE OF CONTENTS

ARQULE, INC.
STATEMENTS OF CASH FLOWS

	YEAR ENDED DECEMBER 31,		
	2018	2017	2016
	(IN THOUSANDS)		
Cash flows from operating activities:			
Net loss	$ (15,482)	$ (29,203)	$ (22,718)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	46	65	101
Amortization of premium (discount) on marketable securities	691	(28)	48
Amortization of debt discount	322	330	—
Change in fair value of warrant liability	1,552	902	—
Non-cash stock compensation	1,503	1,434	1,865
Changes in operating assets and liabilities:			
Contract receivables	(5,984)	—	—
Prepaid expenses and other assets	(314)	322	(108)
Accounts payable and accrued expenses	4,690	(497)	2,466
Deferred revenue	—	1,500	(4,591)
Net cash used in operating activities	(12,976)	(25,175)	(22,937)
Cash flows from investing activities:			
Purchases of marketable securities	(112,580)	(41,971)	(30,975)
Proceeds from sale or maturity of marketable securities	59,295	30,033	39,856
Purchases of property and equipment	—	—	(15)
Net cash provided by (used in) investing activities	(53,285)	(11,938)	8,866
Cash flows from financing activities:			
Proceeds from notes payable and warrants	—	15,000	—
Debt issuance costs associated with notes payable and warrants	(49)	(376)	—
Proceeds from common stock offerings and warrants, net	64,561	17,951	15,174
Proceeds from convertible preferred stock offering and warrants, net	—	9,483	—
Proceeds from stock option exercises and employee stock plan purchases	756	17	181
Net cash provided by financing activities	65,268	42,075	15,355
Net increase (decrease) in cash and cash equivalents	(993)	4,962	1,284
Cash and cash equivalents, beginning of period	20,229	15,267	13,983
Cash and cash equivalents, end of period	$ 19,236	$ 20,229	$ 15,267

SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION (IN THOUSANDS):
We paid interest on debt of $1,344 in 2018, $1,190 in 2017 and $0 in 2016.
We paid no income taxes in 2018, 2017 or 2016.
The accompanying notes are an integral part of these financial statements.

TABLE OF CONTENTS

ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

1. ORGANIZATION AND NATURE OF OPERATIONS

We are a biopharmaceutical company engaged in the research and development of innovative therapeutics to treat cancers and rare diseases. Our mission is to discover, develop and commercialize novel small molecule drugs in areas of high unmet need that will dramatically extend and improve the lives of our patients. These product candidates target biological pathways implicated in a wide range of cancers and certain non-oncology indications. Our discovery and development efforts are guided, when possible, by an understanding of the role of biomarkers, which are indicators of a particular biological condition or process and may predict the clinical benefit of our compounds in defined patient populations. Our clinical-stage pipeline consists of four product candidates, all of which are in targeted patient populations, making ArQule a leader among companies our size in precision medicine.

ArQule has a long history of kinase drug discovery and development, having discovered and introduced ten kinase inhibitors into clinical trials. Our drug discovery efforts have been informed by our historical expertise in chemistry, our work in rational drug design and by our insight into kinase binding and regulation. We have applied this knowledge to produce significant chemical matter for a number of kinase targets and to build an extensive library of proprietary compounds with the potential to target multiple kinases in oncology and other therapeutic areas, such as rare diseases. We may bring further preclinical programs forward and interrogate our library against new targets beyond kinases either directly or with collaborators.

Our proprietary pipeline of orally bioavailable product candidates is directed toward molecular targets and biological processes with demonstrated roles in the development of both human cancers and rare, non-oncology diseases. All of these programs are being developed in targeted, biomarker-defined patient populations. By seeking out subgroups of patients that are most likely to respond to our product candidates, we seek to identify small, often orphan, indications that allow for focused and efficient development. At the same time, in addition to pursuing these potentially fast-to-market strategies, we also pursue development in other indications that could allow us to expand the utility of the product candidates if approved. Our clinical pipeline includes the following product candidates:

- ARQ 531 is a potent and reversible dual inhibitor of both wild type and C481S-mutant Bruton’s tyrosine kinase (BTK) that is in Phase 1 clinical development for B-cell malignancies refractory to other therapeutic options;

- Miransertib (ARQ 092) is a potent and selective inhibitor of protein kinase B (AKT), a serine/ threonine kinase. We expect to commence a registrational clinical trial of miransertib for the treatment of Proteus syndrome and PIK3CA-Related Overgrowth Syndromes (PROS) in the first half of 2019. Miransertib is also in Phase 1b clinical development in oncology in combination with the hormonal therapy, anastrozole;

- ARQ 751 is a next-generation, highly potent and selective inhibitor of AKT that is in Phase 1 clinical development for solid tumors harboring AKT, phosphoinositide 3-kinase (PI3K) or phosphatase and tensin homolog (PTEN) loss mutations; and

- Derazantinib (ARQ 087) is a multi-kinase inhibitor designed to preferentially inhibit the fibroblast growth factor receptor (FGFR) family of kinases that is in a registrational clinical trial in intrahepatic cholangiocarcinoma (iCCA) in patients with FGFR2 fusions. Derazantinib was exclusively licensed to Basilea Pharmaceutica Limited (Basilea) in April 2018 in the United States, European Union, Japan and the rest of the world, excluding the People’s Republic of China, Hong Kong, Macau, and Taiwan (collectively, Greater China) where derazantinib was exclusively licensed to Sinovant Sciences Ltd., a subsidiary of Roivant Sciences Ltd. (Sinovant) in February 2018.

63

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

1. ORGANIZATION AND NATURE OF OPERATIONS (Continued)

Our uses of cash for operating activities have primarily consisted of salaries and wages for our employees, facility and facility-related costs for our offices and laboratories, fees paid in connection with preclinical and clinical studies, laboratory supplies and materials, and professional fees. The sources of our cash flow from operating activities have historically consisted primarily of payments received from our collaborators for services performed or upfront payments for future services. In the year ended December 31, 2018, our net use of cash was primarily driven by the difference between cash received from our collaborations and payments for operating expenses which resulted in net cash outflows of $13.0 million. For the year ended December 31, 2017 net use of cash was primarily driven by payments for operating expenses which resulted in net cash outflows of $25.2 million.

Our cash requirements may vary materially from those now planned depending on the results of our drug discovery and development strategies, our ability to enter into additional corporate collaborations and the terms of such collaborations, results of research and development, unanticipated required capital expenditures, competitive and technological advances, acquisitions and other factors. We cannot guarantee that we will be able to develop any of our product candidates into a commercial product.

In February 2016, we entered into definitive stock purchase agreements with certain institutional and accredited investors. In conjunction with this stock offering we issued 8,027,900 shares of our common stock for aggregate net proceeds of $15.2 million.

In January 2017, we entered into a loan and security agreement with Oxford Finance, LLC (the “Loan Agreement”) with a principal balance of $15 million (see Note 8). The Loan Agreement required interest-only payments for 18 months, followed by an amortization period of 36 months. The original maturity date of the loan was August 1, 2021 and in February 2018 we signed an amendment with the lender which extended the maturity date by one year to August 1, 2022 with principal payments commencing on September 1, 2019.

In September 2017, we sold 2.0 million shares of common stock through an at-the-market (“ATM”) offering and raised net proceeds of approximately $2.3 million.

In October 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering, we issued 13,938,651 shares of our common stock and warrants for 3,123,674 shares of our common stock for aggregate net proceeds of $15.6 million. Each warrant is exercisable at a price of $1.75 per share and expires in October 2021.

In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering we raised net proceeds of $9.5 million through the sale of 8,370 shares of series A convertible preferred stock (Series A Preferred) and warrants to purchase 2,259 shares of Series A Preferred (Warrants). Each share of Series A Preferred converted into 1,000 shares of common stock and each associated Warrant converted into 1,000 common stock warrants upon the effectiveness on May 8, 2018 of an amendment to our restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The Warrants have a post-conversion exercise price of $1.75 per share, are exercisable immediately and expire in May 2022.

In February 2018, we entered into a License Agreement with Sinovant pursuant to which ArQule granted Sinovant an exclusive license to develop and commercialize derazantinib in the People’s Republic of China, Hong Kong, Macau, and Taiwan (collectively, Greater China). The agreement provides for an upfront payment to ArQule of $3 million and a $2.5 million development milestone that was paid in the first quarter of 2019. We are also eligible for up to an additional $82 million in regulatory and sales milestones. Upon commercialization, we are entitled to receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in Greater China. Sinovant will be responsible for all costs and expenses of development, manufacture and commercialization in Greater China.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

1. ORGANIZATION AND NATURE OF OPERATIONS (Continued)

In April 2018, we entered into a License Agreement with Basilea pursuant to which ArQule granted Basilea an exclusive license to develop and commercialize derazantinib in the United States, European Union, Japan and the rest of the world, excluding Greater China. Under the terms of the agreement, we received an upfront payment of $10 million and are eligible for up to $326 million in regulatory and commercial milestones. Upon commercialization, we are entitled to receive staggered royalties on future net sales of derazantinib ranging from the high-single digits to the mid-teens on direct sales and mid-single digits to low-double digits on indirect sales. Basilea will be responsible for all costs and expenses of development, manufacture and commercialization in its territory. Under certain circumstances, we may have the opportunity to promote derazantinib in the United States directly.

In July 2018, we sold 12,650,000 shares of common stock at $5.50 per share for aggregate net proceeds of approximately $64.6 million after commissions and other estimated offering expenses.

We anticipate that our cash, cash equivalents and marketable securities on hand at December 31, 2018, financial support from our licensing agreements and the one year extension of our loan agreement mentioned above will be sufficient to finance our operations for at least 12 months from the issuance date of these financial statements. We expect that we will need to raise additional capital or incur indebtedness to continue to fund our operations in the future. Our ability to raise additional funds will depend on financial, economic and market conditions, and due to global capital and credit market conditions or for other reasons, we may be unable to raise capital when needed, or on terms favorable to us. If necessary funds are not available, we may have to delay, reduce the scope of, or eliminate some of our development programs, potentially delaying the time to market for any of our product candidates.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant accounting policies followed in the preparation of these financial statements are as follows:

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from these estimates.

Cash Equivalents and Marketable Securities

We consider all highly liquid investments purchased within three months of original maturity date to be cash equivalents. We invest our available cash primarily in commercial paper, money market funds and U.S. Treasury bill funds. We generally classify our marketable securities as available-for-sale at the time of purchase and re-evaluate such designation as of each balance sheet date. We classify our investments as either current or long-term based upon the investments' contractual maturities and our ability and intent to convert such instruments to cash within one year. We report available-for-sale investments at fair value as of each balance sheet date and include any unrealized gains and, to the extent deemed temporary, unrealized losses in stockholders' equity. Realized gains and losses are determined using the specific identification method and are included in other income (expense) in the statement of operations and comprehensive loss. For any of our marketable securities classified as trading securities, changes in the fair value of those securities are recorded as other income (expense) in the statement of operations and comprehensive loss. At December 31, 2018 we had no trading securities.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

We conduct quarterly reviews to determine the fair value of our investment portfolio and to identify and evaluate each investment that has an unrealized loss, in accordance with the meaning of other-than- temporary impairment and its application to certain investments. An unrealized loss exists when the current fair value of an individual security is less than its amortized cost basis. In the event that the cost basis of a security exceeds its fair value, we evaluate, among other factors, the duration of the period that, and extent to which, the fair value is less than cost basis, the financial health of and business outlook for the issuer, including industry and sector performance, and operational and financing cash flow factors, overall market conditions and trends, our intent to sell the investment and if it is more likely than not that we would be required to sell the investment before its anticipated recovery. Unrealized losses on available-for- sale securities that are determined to be temporary, and not related to credit loss, are recorded in accumulated other comprehensive income (loss).

For available-for-sale debt securities with unrealized losses, we perform an analysis to assess whether we intend to sell or whether we would more likely than not be required to sell the security before the expected recovery of the amortized cost basis. Where we intend to sell a security, or may be required to do so, the security's decline in fair value is deemed to be other-than-temporary and the full amount of the unrealized loss is reflected in the statement of operations and comprehensive loss as an impairment loss.

Regardless of our intent to sell a security, we perform additional analysis on all securities with unrealized losses to evaluate losses associated with the creditworthiness of the security. We did not recognize any other-than-temporary impairments during the years ended December 31, 2018, 2017 or 2016. Credit losses are identified where we do not expect to receive cash flows sufficient to recover the amortized cost basis of a security.

Fair Value Measurements

Certain of our assets are carried at fair value under U.S. generally accepted accounting principles (GAAP). Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.

- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.

- Level 3—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

At December 31, 2018 and 2017 our financial instruments consisted of cash, cash equivalents, investments in corporate debt securities, accounts payable, notes payable and accrued expenses. At December 31, 2018 and 2017, our financial instruments also included marketable securities which are reported at fair value. At December 31, 2017 our financial instruments also included warrant liabilities reported at fair value. The warrant liability was carried at fair

value and determined to be a Level 3 liability

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

in the fair value hierarchy described above. The carrying values of cash, cash equivalents, investments in corporate debt securities, marketable securities, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities. The carrying value of our notes payable approximates their fair value.

Property and Equipment

Property and equipment are recorded at cost and depreciated using the straight-line method over their estimated useful lives. Assets under capital leases and leasehold improvements are amortized over the shorter of their estimated useful lives or the term of the respective leases by use of the straight-line method. Maintenance and repair costs are expensed as incurred. Depreciation and amortization expense for the years ended December 31, 2018, 2017 and 2016 was $46, $65 and $101, respectively.

Revenue Recognition—Research and Development Revenue

Research and development revenue is generated primarily through collaborative research and development agreements and license agreements. The terms of the agreements may include nonrefundable upfront payments, funding for research and development, milestone payments and royalties on any product sales derived from collaborations.

Our policy is to recognize revenues when our customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. We recognize revenues following the five step model prescribed under Accounting Standards Codification Topic 606—Revenue from Contracts with Customers. Under Topic 606 we: (i) identify contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenues when (or as) we satisfy the performance obligation. We only apply the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, we assess the goods or services promised within each contract, and determine those that are performance obligations. The transaction price is allocated to the identified performance obligations in proportion to their estimated standalone selling prices (SSP) on a relative basis. Determining the estimated SSP for performance obligations requires judgment. In developing the SSP for a performance obligation, we consider applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. Revenue is recognized when each distinct performance obligation is satisfied.

For certain performance obligations that are satisfied over time we recognize revenue using the cost-to-cost method. Under the cost-to-cost method, the extent of progress towards completion is measured based on the ratio of actual costs incurred to the total estimated costs expected upon satisfying the identified performance obligation. Under this method, revenue will be recorded as a percentage of the estimated transaction price based on the extent of progress towards completion. Incurred cost represents work performed, which corresponds with, and thereby best depicts, the transfer of control to the customer. Due to the nature of the work performed in these arrangements, the estimation of cost at completion is subject to many variables and requires judgment. Circumstances can arise that change original estimates of costs or progress toward completion. Changes to the estimated cost at completion have not had a material impact on the results of operations.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

Research and Development Costs
Costs of research and development, which are expensed as incurred, are comprised of the following types of costs incurred in performing research and development activities and those incurred in connection with research and development revenue: salaries and benefits, allocated overhead and occupancy costs, clinical trial and related clinical manufacturing costs, contract services, and other outside costs.

Impairment or Disposal of Long-Lived Assets
We assess our long-lived assets for impairment whenever events or changes in circumstances (a “triggering event”) indicate that the carrying value of a group of long-lived assets may not be recoverable. If such circumstances are determined to exist, an estimate of undiscounted future cash flows produced by the long-lived asset, including its eventual residual value, is compared to the carrying value to determine whether impairment exists. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, the assets are written-down to their estimated fair values.

Segment Data
The chief operating decision maker uses aggregated-financial information in determining how to allocate resources and assess performance. For this reason, we have determined that we are principally engaged in one operating segment. See Note 14 with respect to significant customers. Substantially all of our revenue since inception has been generated in the U.S. and all of our long-lived assets are located in the U.S.

Other Income (Expense)
Other income (expense) in 2018 includes a non-cash expense of $1,552 from an increase in fair value of our preferred stock warrant liability. The preferred stock warrant liability was eliminated upon the conversion of the preferred shares into common shares in the year ended December 31, 2018. Other income (expense) in 2017 includes a non-cash expense of $902 from an increase in fair value of our preferred stock warrant liability. Other income (expense) was $0 in 2016.

Income Taxes
Income taxes have been accounted for using the asset and liability method. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. A valuation allowance against deferred tax assets is recorded if, based upon the weight of all available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. For uncertain tax positions that meet “a more likely than not” threshold, we recognize the benefit of uncertain tax positions in the financial statements.

Earnings (Loss) Per Share
Net loss per share is computed using the weighted average number of common shares outstanding. Basic and diluted net loss per share amounts are equivalent for the periods presented as the inclusion of potential common shares in the number of shares used for the diluted computation would be anti-dilutive to loss per share.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

Potential common shares, for the year ended December 31, 2018, include 10,748,157 shares that would be issued upon the exercise of outstanding employee and Board of Director stock options, 93,168 shares that would be issued upon the exercise of the warrants from the February 2018 amendment to our loan agreement, 3,123,674 shares that would be issued upon the exercise of the warrants from our October 2017 common stock offering, and 2,259,000 common shares that would be issued upon the exercise of the warrants from our November 2017 preferred stock offering. The shares and warrants from our November 2017 preferred stock offering were converted on May 8, 2018 to common shares and warrants. Potential common shares, for the year ended December 31, 2017, include 10,622,455 shares that would be issued upon the exercise of outstanding employee and Board of Director stock options, 354,330 shares that would be issued upon the exercise of the warrants from our January 2017 loan agreement, 3,123,674 shares that would be issued upon the exercise of the warrants from our October 2017 common stock offering, 8,370,000 common shares that would be issued upon the conversion of the shares from our November 2017 preferred stock offering and 2,259,000 common shares that would be issued upon the exercise of the warrants from our November 2017 preferred stock offering. Potential common shares for the year ended December 31, 2016, include 8,715,048 shares that would be issued upon the exercise of outstanding employee and Board of Director stock options and options to purchase 3,567,956 shares that would have been issued upon the exercise of the options from our February 2016 common stock offering. The options issued in conjunction with the February 2016 common stock offering expired in 2017.

Stock-Based Compensation

Our stock-based compensation cost is measured at the grant date, based on the calculated fair value of the award, and is recognized as an expense over the employees' requisite service period (generally the vesting period of the equity grant).

We estimate the fair value of stock options using the Black-Scholes valuation model. Key input assumptions used to estimate the fair value of stock options include the exercise price of the award, expected option term, expected volatility of our stock over the option's expected term, risk-free interest rate over the option's expected term, and the expected annual dividend yield. We believe that the valuation technique and approach utilized to develop the underlying assumptions are appropriate in calculating the fair values of our stock options granted.

The following table presents stock-based compensation expense for the years ended December 31, 2018, 2017 and 2016 included in our Statements of Operations and Comprehensive Loss:

	2018	2017	2016
Research and development	$ 369	$ 359	$ 514
General and administrative	1,134	1,075	1,351
Total stock-based compensation expense	$ 1,503	$ 1,434	$ 1,865

In the years ended December 31, 2018, 2017 and 2016, no stock-based compensation expense was capitalized and there were no recognized tax benefits associated with the stock-based compensation charges.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

The fair value of stock options and employee stock purchase plan shares granted in the years ended December 31, 2018, 2017 and 2016 respectively were estimated on the grant date using the Black-Scholes option-pricing model with the following assumptions:

	2018	2017	2016
Dividend yield(1)	0.0%	0.0%	0.0%
Weighted average expected volatility factor(2)	62%	62%	63%
Risk free interest(3)	2.4–2.9%	1.9–2.1%	1.2–1.8%
Expected term, excluding options issued pursuant to the Employee Stock Purchase Plan(4)	5.6–7.2 years	6.1–7.1 years	5.8–7.2 years
Expected term—Employee Stock Purchase Plan(5)	6 months	6 months	6 months

(1)
We have historically not paid dividends on our common stock and we do not anticipate paying any dividends in the foreseeable future.

(2)
Measured using an average of historical daily price changes of our stock over a period equal to our expected term.

(3)
The risk-free interest rate for periods equal to the expected term of share option based on the U.S. Treasury yield in effect at the time of grant.

(4)
The expected term is the number of years that we estimate, based on historical experience, that options will be outstanding before exercise or cancellation. The range in expected term is the result of certain groups of employees exhibiting different exercising behavior.

(5)
The expected term of options issued in connection with our Employee Stock Purchase Plan is 6 months based on the terms of the plan.

Recent Accounting Pronouncements
From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (“FASB”) or other standard setting bodies that are adopted by us as of the specified effective date. Unless otherwise discussed, we believe that the impact of recently issued standards that are not yet effective will not have a material impact on our financial position or results of operations upon adoption.
In May 2017 the FASB issued Accounting Standard Update (“ASU”) No. 2017-09, Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting. This new standard provides clarity and reduces both (1) diversity in practice and (2) cost and complexity when applying the guidance in Topic 718, Compensation-Stock Compensation, to a change to the terms or conditions of a share-based payment award. This new standard became effective for us on January 1, 2018. The adoption of this standard did not have a material impact on our financial position or results of operations.

In August 2016, the FASB issued ASU No. 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. This new standard clarifies certain aspects of the statement of cash flows, including the classification of debt prepayment or debt extinguishment costs or other debt instruments with coupon interest rates that are insignificant in relation to the effective interest rate of the borrowing, contingent consideration payments made after a business combination, proceeds

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

from the settlement of insurance claims, proceeds from the settlement of corporate-owned life insurance policies, distributions received from equity method investees and beneficial interests in securitization transactions. This new standard also clarifies that an entity should determine each separately identifiable source of use within the cash receipts and payments on the basis of the nature of the underlying cash flows. In situations in which cash receipts and payments have aspects of more than one class of cash flows and cannot be separated by source or use, the appropriate classification should depend on the activity that is likely to be the predominant source or use of cash flows for the item. This new standard became effective for us on January 1, 2018. The adoption of this standard did not have a material impact on our statements of cash flows upon adoption.

In March 2016, the FASB issued ASU No. 2016-09, Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. The new standard involves several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. We adopted this ASU in 2017 and it did not have a material impact on our financial position, results of operations or statement of cash flows.

In February 2016 the FASB issued ASU No. 2016-02, Leases (Topic 842). This standard established a right-of-use model that requires all lessees to recognize right-of-use assets and liabilities on their balance sheet that arise from leases with terms longer than 12 months as well as provide disclosures with respect to certain qualitative and quantitative information related to their leasing arrangements. This standard became effective for us on January 1, 2019.

The FASB has subsequently issued the following amendments to ASU 2016-02, which have the same effective date and transition date of January 1, 2019, and which we collectively refer to as the new leasing standards:

- ASU No. 2018-01, Leases (Topic 842): Land Easement Practical Expedient for Transition to Topic 842, which permits an entity to elect an optional transition practical expedient to not evaluate under Topic 842 land easements that exist or expired prior to adoption of Topic 842 and that were not previously accounted for as leases under the prior standard, ASC 840, Leases.

- ASU No. 2018-10, Codification Improvements to Topic 842, Leases, which amends certain narrow aspects of the guidance issued in ASU 2016-02.

- ASU No. 2018-11, Leases (Topic 842): Targeted Improvements, which allows for a transition approach to initially apply ASU 2016-02 at the adoption date and recognize a cumulative-effect adjustment to the opening balance of accumulated deficit in the period of adoption as well as an additional practical expedient for lessors to not separate non-lease components from the associated lease component.

- ASU No. 2018-20, Narrow-Scope Improvements for Lessors, which contains certain narrow scope improvements to the guidance issued in ASU 2016-02.

We adopted the new leasing standards on January 1, 2019, using a modified retrospective transition approach to be applied to leases existing as of, or entered into after, January 1, 2019. We have reviewed our existing lease contracts and the impact of the new leasing standards on our results of operations, financial position and disclosures. Upon adoption of the new leasing standards, we expect to recognize a lease liability and related right-of-use asset on our

balance sheet of approximately $0.7 million. We expect that the impact of adoption of the new leasing standards will not have a material impact on our statement of income.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

In November 2015, the FASB issued ASU No. 2015-17, Balance Sheet Classification of Deferred Taxes (Topic 740), which requires that deferred tax liabilities and assets be classified as noncurrent in a classified statement of financial position to simplify the presentation of deferred income taxes. This new standard became effective for us on January 1, 2017 and did not have a material impact on our disclosures.

In May 2014 the FASB issued Accounting Standards Update (ASU) No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes all existing revenue recognition requirements, including most industry specific guidance. The FASB subsequently issued amendments to ASU No. 2014-09 that have the same effective date and transition date. This standard requires a company to recognize revenues when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. Under the new revenue standards, we recognize revenues when our customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. We recognize revenues following the five-step model prescribed under Topic 606: (i) identify contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenues when (or as) we satisfy the performance obligation. We only apply the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, we assess the goods or services promised within each contract, and determines those that are performance obligations. The transaction price is allocated to the identified performance obligations in proportion to their estimated standalone selling prices (SSP) on a relative basis. Determining the estimated SSP for performance obligations requires judgment. In developing the SSP for a performance obligation, we consider applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. Revenue is recognized when each distinct performance obligation is satisfied. For certain performance obligations that are satisfied over time the we recognize revenue using the cost-to-cost method. Under the cost-to-cost method, the extent of progress towards completion is measured based on the ratio of actual costs incurred to the total estimated costs expected upon satisfying the identified performance obligation. Under this method, revenue will be recorded as a percentage of the estimated transaction price based on the extent of progress towards completion. Incurred cost represents work performed, which corresponds with, and thereby best depicts, the transfer of control to the customer. Due to the nature of the work performed in these arrangements, the estimation of cost at completion is subject to many variables and requires judgment. Circumstances can arise that change original estimates of costs or progress toward completion. Changes to the estimated cost at completion have not had a material impact on the results of operations.

We adopted Accounting Standards Codification Topic 606—Revenue from Contracts with Customers, or Topic 606, on January 1, 2018, using the modified retrospective approach and resulted in a change to its accounting policy for revenue recognition. Results for reporting periods beginning after January 1, 2018 are presented under the new standard, while prior period amounts are not adjusted and continue to be reported under the accounting standards in effect for the prior period. We recorded a net increase to opening equity of $1.5 million as of January 1, 2018 due to the cumulative impact of adopting this new standard. Without applying the new revenue standard, the disclosed research and development revenue would have been $1.4 million higher than currently disclosed for the year ended December 31, 2018. Contract receivables were $6.0 million at December 31, 2018. The adoption of the new revenue standard did not have a material

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

impact on any other balances within the financial statements as of and for the year ended December 31, 2018. However, the adoption of the new revenue standards will result in a change in the timing of revenue recognition related to certain of our licensing agreements based upon the terms of the underlying agreements.

3. COLLABORATIONS AND ALLIANCES

Basilea Licensing Agreement

In April 2018, we entered into a License Agreement with Basilea pursuant to which ArQule granted Basilea an exclusive license to develop and commercialize derazantinib in the United States, European Union, Japan and the rest of the world, excluding Greater China. Under the terms of the agreement, we received an upfront payment of $10 million and are eligible for up to $326 million in regulatory and commercial milestones. Upon commercialization, we are entitled to receive staggered royalties on future net sales of derazantinib ranging from the high-single digits to the mid-teens on direct sales and mid-single digits to low-double digits on indirect sales. Basilea will be responsible for all costs and expenses of development, manufacture and commercialization in its territory. Under certain circumstances, we may have the opportunity to promote derazantinib in the United States directly.

Revenue in the year ended December 31, 2018 totaled $18.5 million for providing the technology license as well as certain research and development services to Basilea, recognized as revenue on a cost-to-cost method.

Sinovant Licensing Agreement

In February 2018, we entered into a License Agreement with Sinovant pursuant to which ArQule granted Sinovant an exclusive license to develop and commercialize derazantinib in Greater China. The agreement provides for an upfront payment to ArQule of $3 million and a $2.5 million development milestone that was paid in the first quarter of 2019. We are also eligible for up to an additional $82 million in regulatory and sales milestones. Upon commercialization, we are entitled to receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in Greater China. Sinovant will be responsible for all costs and expenses of development, manufacture and commercialization in Greater China. For the year ended December 31, 2018, we recognized revenue of $5.9 million related to the Sinovant agreement, including $0.4 million for certain research and development services that we provided.

Other Licensing Agreements

In October 2017, we entered into a non-exclusive license agreement for certain library compounds. The licensed compounds were delivered and are subject to quality and acceptance testing. In 2017, we recorded deferred revenue of $1.5 million related to this licensing agreement which was recorded as an opening adjustment to accumulated deficit upon the adoption of Accounting Standards Update (ASU) No. 2014-09, Revenue from Contracts with Customers on January 1, 2018. For the year ended December 31, 2018, we recorded revenue of $1.3 million based upon the achievement of the quality and acceptance testing under this completed agreement.

Tivantinib Agreements

We entered into licensing agreements with Daiichi Sankyo and Kyowa Hakko Kirin in December 2008 and May 2007, respectively, related to the development and commercialization of tivantinib. In 2017, we announced that the trials of tivantinib did not meet their primary end points and as a result, we, Daiichi Sankyo and Kyowa Hakko Kirin have all discontinued development of tivantinib.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

4. MARKETABLE SECURITIES AND FAIR VALUE MEASUREMENTS

We generally classify our marketable securities as available-for-sale at the time of purchase and re-evaluate such designation as of each balance sheet date. Since we generally intend to convert them into cash as necessary to meet our liquidity requirements our marketable securities are classified as cash equivalents if the original maturity, from the date of purchase, is ninety days or less and as short-term investments if the original maturity, from the date of purchase, is in excess of ninety days but less than one year. Our marketable securities are classified as long-term investments if the maturity date is in excess of one year of the balance sheet date.

We report available-for-sale investments at fair value as of each balance sheet date and include any unrealized gains and, to the extent deemed temporary, unrealized losses in stockholders' equity. Realized gains and losses are determined using the specific identification method and are included in other income (expense) in the statement of operations and comprehensive loss. We conduct quarterly reviews to determine the fair value of our investment portfolio and to identify and evaluate each investment that has an unrealized loss, in accordance with the meaning of other-than-temporary impairment and its application to certain investments. An unrealized loss exists when the current fair value of an individual security is less than its amortized cost basis. In the event that the cost basis of a security exceeds its fair value, we evaluate, among other factors, the duration of the period that, and extent to which, the fair value is less than cost basis, the financial health of and business outlook for the issuer, including industry and sector performance, and operational and financing cash flow factors, overall market conditions and trends, our intent to sell the investment and if it is more likely than not that we would be required to sell the investment before its anticipated recovery. Unrealized losses on available-for-sale securities that are determined to be temporary, and not related to credit loss, are recorded in accumulated other comprehensive income (loss).

For available-for-sale debt securities with unrealized losses, we perform an analysis to assess whether we intend to sell or whether we would more likely than not be required to sell the security before the expected recovery of the amortized cost basis. Where we intend to sell a security, or may be required to do so, the security's decline in fair value is deemed to be other-than-temporary and the full amount of the unrealized loss is reflected in the statement of operations and comprehensive loss as an impairment loss.

Regardless of our intent to sell a security, we perform additional analysis on all securities with unrealized losses to evaluate losses associated with the creditworthiness of the security. Credit losses are identified where we do not expect to receive cash flows sufficient to recover the amortized cost basis of a security.

We invest our available cash primarily in commercial paper, money market funds, and U.S. Treasury bill funds that have investment grade ratings.

The following is a summary of the fair value of available-for-sale marketable securities we held at December 31, 2018 and December 31, 2017:

December 31, 2018	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Security type				
Corporate debt securities-short term	$ 80,417	$ 2	$ (97)	$ 80,322
Total available-for-sale marketable securities	$ 80,417	$ 2	$ (97)	$ 80,322

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

4. MARKETABLE SECURITIES AND FAIR VALUE MEASUREMENTS (Continued)

December 31, 2017	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Security type				
Corporate debt securities-short term	$ 27,823	$ 1	$ (17)	$ 27,807
Total available-for-sale marketable securities	$ 27,823	$ 1	$ (17)	$ 27,807

None of our available-for-sale marketable securities were in a continuous unrealized loss position for more than 12 months at December 31, 2018 or 2017.

The following table presents information about our assets that are measured at fair value on a recurring basis for the year ended December 31, 2018 and indicates the fair value hierarchy of the valuation techniques we utilized to determine such fair value. There were no transfers in or out of Level 1 or Level 2 measurements for the year ended December 31, 2018:

	December 31, 2018	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents	$ 14,444	$ 14,444	$ —	$ —
Corporate debt securities-short term	80,322	—	80,322	—
Total	$ 94,766	$ 14,444	$ 80,322	$ —

The following tables present information about our assets and liabilities that are measured at fair value on a recurring basis for the year ended December 31, 2017 and indicates the fair value hierarchy of the valuation techniques we utilized to determine such fair value. There were no transfers in or out of Level 1 or Level 2 measurements for the year ended December 31, 2017:

	December 31, 2017	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents	$ 19,889	$ 19,889	$ —	$ —
Corporate debt securities-short term	27,807	—	27,807	—
Total	$ 47,696	$ 19,889	$ 27,807	$ —

December 31, 2017	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)

Warrant liability	$ 1,512	$ —	$ —	$ 1,512

Due to the lack of market quotes relating to our preferred stock warrants, the fair value of the preferred stock warrants was determined at December 31, 2017 using the Black-Scholes model, which is based on Level 3 inputs. As of December 31, 2017, inputs used in the Black-Scholes model are presented below. Based on the Black-Scholes model, we recorded a preferred stock warrants liability of $1,512 at December 31, 2017. Upon conversion of the Series A Preferred to common stock on May 8, 2018 the warrant liability of $3,064 was extinguished with an offsetting amount included as additional paid-in capital in stockholders' equity.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

4. MARKETABLE SECURITIES AND FAIR VALUE MEASUREMENTS (Continued)

The following are the Black-Scholes inputs to the warrant liability valuation at December 31, 2017:

	2017
Warrant stock price	$1.75
Exercise price	1.65
Expected volatility	53.3%
Risk-free interest	2.07%
Expected term	3.85 years
Dividends	none

The following is a summary of the activity in the fair value of our warrant liability for the year ended December 31, 2018:

	Amount
Warrant liability as of December 31, 2017	$ 1,512
Change in fair value	1,552
Conversion of preferred stock warrants to common stock warrants	(3,064)
Warrant liability as of December 31, 2018	$ —

5. PROPERTY AND EQUIPMENT

Property and equipment consist of the following at December 31, 2018 and 2017:

	USEFUL LIFE ESTIMATED (YEARS)	2018	2017
Machinery and equipment	5	$ 1,611	$ 1,939
Leasehold improvements	3–5	232	232
Furniture and fixtures	7	40	40
Computer equipment	3	2,496	2,497
		4,379	4,708
Less: Accumulated depreciation and amortization		4,310	4,593
Net property and equipment		$ 69	$ 115

6. OTHER ASSETS

Other assets include the following at December 31, 2018 and 2017:

	2018	2017
Security deposits	$ 204	$ 204
Total other assets	$ 204	$ 204

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

7. ACCOUNTS PAYABLE AND ACCRUED EXPENSES

Accounts payable and accrued expenses include the following at December 31, 2018 and 2017:

	2018	2017
Accounts payable	$ 1,329	$ 537
Accrued payroll	1,971	1,448
Accrued outsourced preclinical and clinical fees	8,497	5,409
Accrued professional fees	666	492
Other accrued expenses	485	373
	$ 12,948	$ 8,259

8. LOAN AGREEMENT

In January 2017, Oxford Finance LLC, as collateral agent and a lender (the “Lender”), and any additional lenders that may become parties thereto, entered into a loan and security agreement with us (the “Loan Agreement”).

Pursuant to the terms of the Loan Agreement, the Lender issued us a loan in the principal amount of $15.0 million. The loan will bear interest at the rate equal to (a) the greater of (i) the 30 day U.S. LIBOR rate reported in the Wall Street Journal on the date occurring on the last business day of the month that immediately precedes the month in which the interest will accrue or (ii) 0.65% (b) plus 6.85%. The applicable interest rate on the loan at December 31, 2018 was 9.20%. The Loan Agreement required interest-only payments for 18 months, followed by an amortization period of 36 months. The original maturity date of the loan was August 1, 2021 and in February 2018 we signed an amendment with the lender which extended the maturity date by one year to August 1, 2022 with principal payments commencing on September 1, 2019.

The expected remaining repayment of the $15 million loan principal at December 31, 2018 is as follows:

2019	$ 1,667
2020	5,000
2021	5,000
2022	3,333
	$ 15,000

Upon the earlier of prepayment or the maturity date, we will pay to the Lender a final payment of 6% of the full principal amount of the loan. We may elect to prepay all amounts owed prior to the maturity date, provided that a prepayment fee also is paid equal to 1% of the full principal amount of the loan.

Pursuant to the terms of the Loan Agreement, we are bound by certain affirmative covenants setting forth actions that are required during the term of the Loan Agreement, including, without limitation, certain information delivery requirements, obligations to maintain certain insurance, and certain notice requirements. Additionally, we are bound by certain negative covenants setting forth actions that are not permitted to be taken during the term of the Loan Agreement without consent, including, without limitation, incurring certain additional indebtedness, entering into certain mergers, acquisitions or other business combination transactions, or incurring any non-permitted lien or other encumbrance on our assets. We are in compliance with the loan covenants at December 31, 2018.

Upon the occurrence of an event of default under the Loan Agreement (subject to cure periods for certain events of default), all amounts owed by us thereunder will begin to bear interest at a rate that is 5% higher than the rate that is otherwise applicable and may be declared immediately due and payable by the

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

8. LOAN AGREEMENT (Continued)

Lender. Events of default under the Loan Agreement include, among other things, the following: the occurrence of certain bankruptcy events; the failure to make payments under the Loan Agreement when due; the occurrence of a material adverse change in our business, operations or financial condition; the rendering of certain types of fines or judgments against us; any breach by us of any covenant (subject to cure for certain covenants only) made in the Loan Agreement; and the failure of any representation or warranty made by us in connection with the Loan Agreement to be correct in all material respects when made.

We have granted Lender, a security interest in substantially all of our personal property, rights and assets, other than intellectual property, to secure the payment of all amounts owed to the Lender under the Loan Agreement. We have also agreed not to encumber any of our intellectual property without required lenders' prior written consent.

In connection with entering into the Loan Agreement, we issued to the Lender warrants to purchase an aggregate of 354,330 shares of our common stock (the "Lender Warrants"). The warrants are exercisable immediately, have a per-share exercise price of $1.27 and have a term of ten years. We have recorded the relative fair value of the warrants as a discount to the carrying value of the notes payable with a corresponding increase to additional paid in capital.

In February 2018, the Loan Agreement was amended requiring payments of interest on a monthly basis through August 2019 and payments of interest and principal from September 2019 to August 2022. In connection with entering into the amendment we issued to the Lender warrants to purchase an aggregate of 93,168 shares of our common stock. The warrants are exercisable immediately, have a per-share exercise price of $1.61 and have a term of ten years. The amendment was determined a modification of debt according to ASC 470 Debt.

9. PREFERRED STOCK AND WARRANT LIABILITY

Our amended Certificate of Incorporation authorizes the issuance of up to 1 million shares of $0.01 par value preferred stock.

In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering we raised net proceeds of $9.5 million through the sale of 8,370 shares of series A convertible preferred stock (Series A Preferred) and warrants covering 2,259 shares of Series A Preferred (Warrants). Each share of Series A Preferred converted into 1,000 shares of common stock and each associated Warrant converted into 1,000 common stock warrants upon the effectiveness on May 8, 2018 of an amendment to our restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The amount reported as preferred stock at December 31, 2018 is zero and at December 31, 2017 is $8.8 million. At December 31, 2017 the fair value of the warrant liability increased to $1.5 million and consequently a $0.9 million non-cash expense was recorded.

The terms of the Series A Preferred, specifically the terms of the liquidation preference, required the classification of the preferred stock as temporary equity, which is reflected in our balance sheet as of December 31, 2017. In addition, the terms of the Series A Preferred for which the warrants are exercisable require that the fair value allocated to the warrants at the date of issuance be recorded as a liability. The warrant liability was marked to market value through the income statement as a non-cash gain or loss at each reporting period until the conversion of the preferred stock to common stock on May 8, 2018. The Warrants had a pre-conversion exercise price of $1,750 per share of Series A Preferred (post-conversion price of $1.75 per share of common stock), were exercisable immediately with an expiration date in May 2022. Upon conversion of the Series A Preferred common on May 8, 2018, the warrant liability of $3,064 was extinguished with an offsetting amount included as additional paid-in capital in stockholders' equity.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

9. PREFERRED STOCK AND WARRANT LIABILITY (Continued)

At December 31, 2017 the fair value of the warrant liability was $1.5 million. In the year ended December 31, 2018 the fair value of the warrant liability increased by $1.5 million and non-cash expense was recorded in other expense. At December 31, 2018 the warrant liability was zero.

10. COMMON STOCK

Our amended Certificate of Incorporation authorizes the issuance of up to 200 million shares of common stock, par value $0.01 per share.

At December 31, 2018, we have 4,610,616 shares reserved for future issuance of common stock options pursuant to the 2014 Equity Incentives Plan ("Equity Incentives Plan").

In July 2018, we sold 12,650,000 shares of common stock at $5.50 per share for aggregate net proceeds of approximately $64.6 million after commissions and other estimated offering expenses.

In October 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering, we issued 13,938,651 shares of our common stock and warrants for 3,123,674 shares of our common stock for aggregate net proceeds of $15.6 million. Each warrant is exercisable for $1.75 per share and expires in in October 2021.

In September 2017, we sold 2.0 million shares of common stock through an at-the-market (ATM) offering and raised net proceeds of approximately $2.3 million.

In February 2016, we entered into definitive stock purchase agreements with certain institutional and accredited investors. In conjunction with this stock offering we issued 8,027,900 shares of our common stock and non-transferable options for 3,567,956 shares of our common stock for aggregate net proceeds of $15.2 million. Each option was exercisable for $2.50 per share and they all expired in March 2017.

11. EQUITY INCENTIVE PLANS

In 2014, our stockholders approved our 2014 Equity Incentives Plan and authorized 3,750,000 shares of common stock for issuance pursuant to future awards under that plan. In addition, any shares from our Amended and Restated 1994 Equity Incentive Plan that expire are cancelled or forfeited after the effective date of the 2014 Equity Incentives Plan may also be issued for future awards under the 2014 Equity Incentives Plan. In 2018, our stockholders approved an amendment to our 2014 Equity Incentives Plan to increase the number of shares of common stock available for issuance for issuance pursuant to future awards made under that plan by 3,750,000. All shares are awarded at the discretion of our Board of Directors in a variety of stock based forms including stock options, restricted stock and performance based stock units, and stock appreciation rights. Pursuant to the 2014 Equity Incentives Plan, incentive stock options may not be granted at less than the fair market value of our common stock at the date of the grant, and the option term may not exceed ten years. Stock options issued pursuant to the 2014 Equity Incentives Plan generally vest over four years. For holders of 10% or more of our voting stock, options may not be granted at less than 110% of the fair market value of the common stock at the date of the grant, and the option term may not exceed five years. Stock appreciation rights granted in tandem with an option shall have an exercise price not less than the exercise price of the related option. As of December 31, 2018, no stock appreciation rights have been issued. At December 31, 2018, there were 4,610,616 shares available for future grants under the 2014 Equity Incentives Plan.

During 2014, our stockholders approved an amendment to the 1996 Director Stock Option Plan to increase the number of shares available to 1,200,000. Under the terms of the 1996 Director Stock Option Plan, options to purchase shares of common stock are automatically granted (A) to the Chairman of the Board of Directors (1) upon his or her initial election or appointment in the amount of 25,000 and vesting

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

11. EQUITY INCENTIVE PLANS (Continued)

over three years and (2) upon his or her re-election or continuation on our board immediately after each annual meeting of stockholders in the amount of 25,000 and vesting one year from the date of grant, and (B) to each other Director (1) upon his or her initial election to our board in the amount of 30,000 and vesting over three years and (2) upon his or her re-election or continuation on our board in the amount of 20,000 and vesting one year from the date of grant. All options granted pursuant to the 1996 Director Plan have a term of ten years with exercise prices equal to fair market value on the date of grant. At December 31, 2017, options to purchase 760,000 shares of common stock are outstanding and exercisable under the 1996 Director Plan which terminated in 2016. Additionally, under the 2014 Equity Incentives Plan options issued to Directors to purchase 425,000 shares of common stock are outstanding at December 31, 2018 of which 260,000 are exercisable.

Option activity under the Plans for the year ended December 31, 2018 was as follows:

Stock Options	Number of Shares	Weighted Average Exercise Price
Outstanding as of December 31, 2017	10,622,455	$ 3.01
Granted	1,911,770	2.78
Exercised	(1,104,706)	3.22
Cancelled	(681,362)	3.71
Outstanding as of December 31, 2018	10,748,157	$ 2.90
Exercisable as of December 31, 2018	5,973,658	$ 3.71

The following table summarizes information about options outstanding at December 31, 2018:

	Options Outstanding			Options Exercisable	
Range of Exercise Prices	Number Outstanding at December 31, 2018	Weighted Average Remaining Contractual Life	Weighted Average Exercise Price	Exercisable as of December 31, 2018	Weighted Average Exercise Price
$0.95–1.41	2,352,567	7.46	$ 1.05	753,988	$ 1.17
1.42–2.35	3,329,745	7.71	1.69	1,234,325	1.71
2.36–3.80	2,636,207	4.96	2.73	2,001,207	2.80
3.81–5.60	626,750	7.07	4.81	186,250	4.12
5.61–8.40	1,802,888	2.56	7.13	1,797,888	7.13
	10,748,157	6.08	$ 2.90	5,973,658	$ 3.71

The aggregate intrinsic value of options outstanding at December 31, 2018 was $8,112. The weighted average grant date fair value of options granted in year ended December 31, 2018, 2017 and 2016 was $1.73, $0.72, and $1.10, per share, respectively. In the year ended December 31, 2018 we had 1,104,706 options were exercised. In the year ended December 31, 2017 no options were exercised.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

11. EQUITY INCENTIVE PLANS (Continued)

Options vested, expected to vest and exercisable at December 31, 2018 are as follows:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Vested and unvested expected to vest at December 31, 2018	10,560,209	$ 2.90	6.08	$ 8,112
Exercisable at December 31, 2018	5,973,658	$ 3.71	4.26	$ 2,823

The total compensation cost not yet recognized as of December 31, 2018 related to non-vested option awards was $3,951 which will be recognized over a weighted-average period of 2.7 years. During the year ended December 31, 2018, 251,275 shares were forfeited with a weighted average grant date fair value of $0.81 per share and a weighted average exercise price of $1.36 per share. During the year ended December 31, 2018, 430,087 shares expired with a weighted average grant date fair value of $2.82 per share and a weighted average exercise price of $5.08 per share. The weighted average remaining contractual life for options exercisable at December 31, 2018 was 4.3 years.

In April 2017, we amended our chief executive officer's (the "CEO's") and chief operating officer's (the "COO's") employment agreements to extend the term and also granted them a maximum of 600,000 and 300,000 performance-based stock options, respectively, that vest upon the achievement of certain performance and market-based targets. In April 2017, we amended our chief medical officer's (the "CMO's") employment agreement to extend the term and also granted him 260,000 performance-based stock options that vest upon the achievement of certain performance-based targets. In April 2017, certain other employees were granted a total of 270,000 performance-based stock options that vest upon the achievement of certain performance-based targets 110,000 of these options were forfeited in 2018. Through December 31, 2018 no expense has been recorded for any performance-based stock options granted to the CEO, COO, CMO, or to any other employees.

In 1996, the stockholders adopted the 1996 Employee Stock Purchase Plan. This plan enables eligible employees to exercise rights to purchase our common stock at 85% of the fair market value of the stock on the date the right was granted or the date the right is exercised, whichever is lower. Rights to purchase shares under the 1996 Employee Stock Purchase Plan are granted by the Board of Directors. The rights are exercisable during a period determined by the Board of Directors; however, in no event will the period be longer than twenty-seven months. The 1996 Employee Stock Purchase Plan is available to substantially all employees, subject to certain limitations. In 2011, our stockholders approved an amendment to the Purchase Plan to increase the aggregate number of shares of our common stock that may be issued to 2,400,000. As of December 31, 2017, 2,217,705 shares have been purchased and no shares are available under this plan which was terminated in 2016. In 2018, the stockholders adopted the 2018 Employee Stock Purchase Plan. This plan enables eligible employees to exercise rights to purchase our common stock at 85% of the fair market value of the stock on the date the right was granted or the date the right is exercised, whichever is lower. Rights to purchase shares under the 2018 Employee Stock Purchase Plan are granted by the Board of Directors. We recognized share-based compensation expense related to the 1996 Employee Stock Purchase Plan of $12, $17 and $60 for the year ended December 31, 2018, 2017 and 2016, respectively.

12. INCOME TAXES

There was no current or deferred tax expense for the years ended December 31, 2018, 2017 or 2016 due to our loss before income taxes and our valuation allowance. We have recorded a full valuation allowance against our deferred tax assets based upon the weight of available evidence, as it is more likely than not that the deferred tax assets will not be realized.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

12. INCOME TAXES (Continued)

The following is a reconciliation between the U.S. federal statutory rate and the effective tax rate for the years ended December 31, 2018, 2017 and 2016:

	2018	2017	2016
Income tax (benefit) expense at statutory rate	$ (3,251)	$ (9,929)	$ (7,724)
State tax (benefit) expense, net of Federal tax (benefit) expense	(938)	(1,452)	(1,146)
Permanent items	75	508	263
Effect of change in valuation allowance and State NOL expiration	4,542	(39,972)	8,837
Tax credits	(132)	(544)	(228)
Change in tax rate on beginning assets	—	51,445	—
Other	(296)	(56)	(2)
Tax expense (benefit)	$ —	$ —	$ —

The income tax effect of temporary differences comprising the deferred tax assets and deferred tax liabilities on the accompanying balance sheets is a result of the following at December 31, 2018 and 2017:

	2018	2017
Deferred tax assets:		
Net operating loss carryforwards	$ 103,855	$ 100,421
Tax credit carryforwards	26,880	26,771
Equity based compensation	4,361	3,381
Book depreciation in excess of tax	56	49
Reserves and accruals	178	171
Other	21	16
	135,351	130,809
Valuation allowance	(135,351)	(130,809)
Deferred tax liabilities	—	—
Net deferred tax assets	$ —	$ —

Total valuation allowance increased by $4,542 for the year ended December 31, 2018, decreased by $39,972 for the year ended December 31, 2017 and increased by $8,837 for the year ended December 31, 2016. We have evaluated positive and negative evidence bearing upon the realizability of our deferred tax assets, which are comprised principally of federal and state net operating loss (“NOL”), net capital loss, and research and development credit carryforwards. We have determined that it is more likely than not that we will not recognize the benefits of our federal and state deferred tax assets and, as a result, we have established a full valuation allowance against our net deferred tax assets as of December 31, 2018.

The Tax Cuts and Jobs Act was enacted on December 22, 2017. The Act reduced the US federal corporate tax rate from 35% to 21%, required companies to re-measure the deferred tax assets and deferred tax liabilities as of the date of enactment. In the year ended December 31, 2017, we re-measured deferred tax assets and liabilities based on the rates at which they are expected to reverse in the future, which is generally 21%. The amount recorded related to the

re-measurement of our deferred tax balance before Valuation Allowance was $51,445 which was offset by the full valuation allowance in the year ended December 31, 2017. We determined for the year ended December 31, 2018 that no further adjustment was necessary.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

12. INCOME TAXES (Continued)

As of December 31, 2018, we had federal NOL, state NOL, and research and development credit carryforwards of approximately $422,045, $240,916 and $28,378 respectively, which expire at various dates through 2037. Out of the total federal NOL of $422,045, $12,636 of NOL generated in 2018 has an indefinite life.

As of December 31, 2018, and 2017 we had no unrecognized tax benefits. We do not expect that the total amount of unrecognized tax benefits will significantly increase in the next twelve months. Our policy is to recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2018 and 2017, we had no accrued interest or penalties related to uncertain tax positions. Our U.S. federal tax returns for the tax years 2015 through 2018 and our state tax returns for the tax years 2015 through 2018 remain open to examination. Prior tax years remain open to the extent of net operating loss and tax credit carryforwards.

Utilization of NOL and research and development credit carryforwards may be subject to a substantial annual limitation in the event of an ownership change that has occurred previously or could occur in the future pursuant to Section 382 and 383 of the Internal Revenue Code of 1986, as amended, as well as similar state provisions. An ownership change may limit the amount of NOL and research and development credit carryforwards that can be utilized annually to offset future taxable income, and may, in turn, result in the expiration of a portion of those carryforwards before utilization. In general, an ownership change, as defined by Section 382, results from transactions that increase the ownership of certain stockholders or public groups in the stock of a corporation by more than 50 percentage points over a three-year period. We undertook a detailed study of our NOL and research and development credit carryforwards through January 31, 2019, to determine whether such amounts are likely to be limited by Sections 382 or 383. As a result of this analysis, we currently do not believe any Sections 382 or 383 limitations will significantly impact our ability to offset income with available NOL and research and development credit carryforwards. However, future ownership changes under Section 382 may limit our ability to fully utilize these tax benefits.

13. COMMITMENTS AND CONTINGENCIES

Leases

We lease a facility under a non-cancelable operating lease that terminates on July 31, 2020 and the minimum lease commitment for our leased facility is as follows:

YEAR ENDING DECEMBER 31,	OPERATING LEASES
2019	$ 523
2020	296
Thereafter	—
Total minimum lease payments	$ 819

Rent expense under our non-cancelable operating lease was approximately $554, $546, and $540 for the years ended December 31, 2018, 2017 and 2016, respectively.

In January 2015, we entered into a lease agreement for our headquarters facility. The lease commenced on May 1, 2015 for a term of five years and three months with an average annual rental rate of $455. The lease obligation for the new facility is included in the table above.

TABLE OF CONTENTS
ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)

14. CONCENTRATION OF CREDIT RISK

Revenue from one customer represented approximately 72% of total revenue during 2018 and revenue from another customer represented approximately 23% of total revenue. There were no revenues recognized in 2017. Revenue from one customer represented approximately 40% of total revenue during 2016 and revenue from another customer represented approximately 60% of total revenue.

15. SELECTED QUARTERLY FINANCIAL DATA (UNAUDITED)

	FIRST QUARTER	SECOND QUARTER	THIRD QUARTER	FOURTH QUARTER
2018				
Net revenues	$ 4,138	$ 13,706	$ 4,979	$ 2,941
Net income (loss)	(6,532)	5,156	(5,619)	(8,487)
Basic and diluted net income (loss) per share:				
Basic net income (loss) per share	$ (0.07)	$ 0.06	$ (0.05)	$ (0.08)
Diluted net income (loss) per share	$ (0.07)	$ 0.05	$ (0.05)	$ (0.08)

	FIRST QUARTER	SECOND QUARTER	THIRD QUARTER	FOURTH QUARTER
2017				
Net revenues	$ —	$ —	$ —	$ —
Net loss	(7,576)	(7,201)	(6,666)	(7,760)
Basic and diluted net loss per share:				
Net loss per share	$ (0.11)	$ (0.10)	$ (0.09)	$ (0.09)

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

None.

ITEM 9A.
CONTROLS AND PROCEDURES

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and President and Chief Operating Officer (Principal Financial Officer), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2018. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (“Exchange Act”), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to provide reasonable assurance that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of

our disclosure controls and procedures as of December 31, 2018, our Chief Executive Officer and President and Chief Operating Officer (Principal Financial Officer) concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

TABLE OF CONTENTS

Management's Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rule 13a-15(f). Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in Internal Control—Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under the framework in Internal Control—Integrated Framework (2013), our management concluded that our internal control over financial reporting was effective as of December 31, 2018.

The effectiveness of our internal control over financial reporting as of December 31, 2018 has been audited by PricewaterhouseCoopers LLP, an independent registered public accounting firm, as stated in their report which is included herein.

Changes in Internal Control Over Financial Reporting

There has been no change in our internal control over financial reporting during the year ended December 31, 2018 that has materially affected or is reasonably likely to materially affect our internal control over financial reporting.

ITEM 9B.

OTHER INFORMATION

None.

TABLE OF CONTENTS

PART III

Except as otherwise indicated, the following information required by the Instructions to Form 10-K is incorporated herein by reference from various sections of the ArQule, Inc. Proxy Statement for the annual meeting of stockholders to be held on May 14, 2019, as summarized below:

ITEM 10.

DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE

"Election of Directors;" "Section 16(a) Beneficial Ownership Reporting Compliance;" "Corporate Governance Guidelines and Code of Conduct;" and "Board Committees and Meetings."

Information regarding our executive officers is incorporated by reference from "Executive Officers" at the end of Item 1 of this report.

ITEM 11.

EXECUTIVE COMPENSATION

"Compensation Discussion and Analysis;" "Executive Compensation;" "Director Compensation;" "Compensation Committee Interlocks and Insider Participation;" and "Compensation Committee Report."

ITEM 12.

SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

"Share Ownership of Certain Beneficial Owners" and "Securities Authorized for Issuance Under Equity Compensation Plans."

ITEM 13.

CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

"Certain Relationships and Related Transactions" and "Director Independence."

ITEM 14.

PRINCIPAL ACCOUNTING FEES AND SERVICES

Fees paid to our independent registered public accounting firm are disclosed under the caption "Ratification of the Selection of an Independent Registered Public Accountants."

PART IV

ITEM 15.

EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) 1. FINANCIAL STATEMENTS

The financial statements are listed under Item 8 of this report.

2. FINANCIAL STATEMENT SCHEDULES

The financial statement schedules are omitted from this report because they are not applicable or required information are shown in the financial statements of the footnotes thereto.

TABLE OF CONTENTS

3. EXHIBITS

EXHIBIT NO.	DESCRIPTION
1.1	Capital on Demand™ Sales Agreement, dated October 25, 2016, by and between the Company and JonesTrading Institutional Services LLC. Filed as Exhibit 1.1 to the Company's Current Report on Form 8-K filed on October 25, 2016 (File No. 000-21429) and incorporated herein by reference.
3.1	Restated Certificate of Incorporation of the Company, filed herewith.
3.2	Amended and Restated By-laws of the Company. Filed as Exhibit 3.1 to the Company's Current Report on Form 8-K filed on January 27, 2014 (File No. 000-21429) and incorporated herein by reference.
4.1	Specimen Common Stock Certificate. Filed as Exhibit 4.2 to the Company's Registration Statement on Form S-1 filed on August 19, 1996 (File No. 333-11105) and incorporated herein by reference.
4.2	Form of Common Stock Warrant. Filed as Exhibit 4.1 to the Company's Current Report on Form 8-K filed on October 16, 2017 (File No. 000-21429) and incorporated herein by reference.
4.3	Warrant dated February 16, 2018 issued to Oxford Finance LLC. Filed as Exhibit 4.1 to the Company's Current Report on Form 8-K filed on February 22, 2018 (File No. 000-21429) and incorporated herein by reference.
10.1*	Amended and Restated 1994 Equity Incentive Plan. Filed as Appendix A to the Company's Definitive Proxy Statement filed on April 29, 2011 (File No. 000-21429) and incorporated herein by reference.
10.2*	2018 Employee Stock Purchase Plan. Filed as Appendix B to the Company's Definitive Proxy Statement filed on March 29, 2018 (File No. 000-21429) and incorporated herein by reference.
10.3*	Amended and Restated 1996 Director Stock Option Plan. Filed as Appendix C to the Company's Definitive Proxy Statement filed on April 29, 2011 (File No. 000-21429) and incorporated herein by reference.
10.4*	Employment Agreement between the Company and Peter S. Lawrence dated April 13, 2006. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K dated April 18, 2006 (File No. 000-21429) and incorporated herein by reference.
10.5*	Amendment to Employment Agreement, dated as of October 4, 2007, by and between the Company and Peter S. Lawrence. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on October 10, 2007 (File No. 000-21429) and incorporated herein by reference.
10.6*	Form of Incentive Stock Option Agreement to the Company's Amended and Restated 1994 Equity Incentive Plan. Filed as Exhibit 10.16 to the Company's Annual Report on Form 10-K filed on March 17, 2008 (File No. 000-21429) and incorporated herein by reference.
10.7*	Form of Non-Statutory Stock Option Agreement to the Company's Amended and Restated 1994 Equity Incentive Plan. Filed as Exhibit 10.17 to the Company's Annual Report on Form 10-K filed on March 17, 2008 (File No. 000-21429) and incorporated herein by reference.
10.8*	Second Amendment to Employment Agreement, dated April 14, 2008, by and between ArQule, Inc. and Peter S. Lawrence. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K, filed on April 18, 2008 (File No. 000-21429) and incorporated herein by reference.
10.9*	Employment Agreement, dated as of April 15, 2008, by and between ArQule, Inc. and Paolo Pucci. Filed as Exhibit 10.4 to the Company's Current Report on Form 8-K, filed on April 18, 2008 (File No. 000-21429) and incorporated herein by reference.
10.10*	Amendment to Employment Agreement, dated as of July 15, 2010, by and between the Company and Paolo Pucci. Filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2010 filed on August 4, 2010 (File No. 000-21429) and incorporated herein

by reference.

TABLE OF CONTENTS

EXHIBIT NO.	DESCRIPTION
10.11*	Form of Stock Unit Agreement. Filed as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2010, filed on August 4, 2010 (File No. 000-21429) and incorporated herein by reference.
10.12*	Form of Restricted Stock Agreement. Filed as Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2010, filed on August 4, 2010 (File No. 000-21429) and incorporated herein by reference.
10.13*	Employment Agreement, dated as of June 17, 2008, by and between ArQule, Inc. and Brian Schwartz, Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on February 24, 2012 (File No. 000-21429) and incorporated herein by reference.
10.14*	Amendment to Employment Agreement dated as of February 23, 2012 by and between ArQule, Inc. and Brian Schwartz. Filed as Exhibit 10.2 to Amendment No. 1 to the Company's Current Report on Form 8-K filed on February 27, 2012 (File No. 000-21429) and incorporated herein by reference.
10.15*	Second Amendment to Employment Agreement, dated as of March 8, 2013, by and between ArQule, Inc. and Paolo Pucci. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on March 11, 2013 (File No. 000-21429) and incorporated herein by reference.
10.16*	Third Amendment to Employment Agreement, dated as of March 8, 2013, by and between ArQule, Inc. and Peter S. Lawrence. Filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed on March 11, 2013 (File No. 000-21429) and incorporated herein by reference.
10.17*	Second Amendment to Employment Agreement, dated March 8, 2013, by and between ArQule, Inc. and Brian Schwartz. Filed as Exhibit 10.3 to the Company's Current Report on Form 8-K filed on March 11, 2013 (File No. 000-21429) and incorporated herein by reference.
10.18*	2014 Equity Incentives Plan. Filed as Appendix C to the Company's Definitive Proxy Statement filed on March 29, 2018 (File No. 000-21429) and incorporated herein by reference.
10.19*	Third Amendment to Employment Agreement dated as of April 14, 2016, by and between ArQule, Inc. and Paolo Pucci. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on April 14, 2016 (File No. 000-21429) and incorporated herein by reference.
10.20*	Fourth Amendment to Employment Agreement dated as of April 14, 2016, by and between ArQule, Inc. and Peter S. Lawrence. Filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed on April 14, 2016 (File No. 000-21429) and incorporated herein by reference.
10.21*	Third Amendment to Employment Agreement dated as of April 14, 2016, by and between ArQule, Inc. and Brian Schwartz. Filed as Exhibit 10.3 to the Company's Current Report on Form 8-K filed on April 14, 2016 (File No. 000-21429) and incorporated herein by reference.
10.22	Loan and Security Agreement between and among ArQule, Inc. and Oxford Finance LLC, as Lender, dated January 6, 2017 Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on January 10, 2017 (File No. 000-21429) and incorporated herein by reference.
10.23*	Form of Performance-based Option Agreement to the Company's 2014 Equity Incentives Plan. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on April 10, 2017 (File No. 000-21429) and incorporated herein by reference.
10.24*	Form of Incentive Stock Option Agreement to the Company's 2014 Equity Incentives Plan, filed herewith.
10.25*	Form of Non-Statutory Stock Option Agreement to the Company's 2014 Equity Incentives Plan, filed herewith.
10.26*	

	Fourth Amendment to Employment Agreement, dated as of April 4, 2017 by and between ArQule, Inc. and Paolo Pucci. Filed as Exhibit 10.2 to the Company's Current Report on Form 8-K filed on April 10, 2017 (File No. 000-21429) and incorporated herein by reference.
10.27*	Fifth Amendment to Employment Agreement, dated as of April 4, 2017, by and between ArQule, Inc. and Peter S. Lawrence. Filed as Exhibit 10.3 to the Company's Current Report on Form 8-K filed on April 10, 2017 (File No. 000-21429) and incorporated herein by reference.

TABLE OF CONTENTS

EXHIBIT NO.	DESCRIPTION
10.28*	Fourth Amendment to Employment Agreement, dated as of April 4, 2017, by and between ArQule, Inc. and Brian Schwartz. Filed as Exhibit 10.4 to the Company's Current Report on Form 8-K filed on April 10, 2017 (File No. 000-21429) and incorporated herein by reference.
10.29+	Master Services Agreement, dated July 20, 2017, by and between the Company and ARUP Laboratories, Inc. Filed as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed on November 9, 2017 (File No. 000-21429) and incorporated herein by reference.
10.30+	Scope of Work #1 to Master Services Agreement, dated July 20, 2017, by and between the Company and ARUP Laboratories, Inc. Filed as Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed on November 9, 2017 (File No. 000-21429) and incorporated herein by reference.
10.31+	Scope of Work #2 to Master Services Agreement, dated July 20, 2017, by and between the Company and ARUP Laboratories, Inc. Filed as Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q filed on November 9, 2017 (File No. 000-21429) and incorporated herein by reference.
10.32	Second Amendment to Loan and Security Agreement between and among ArQule, Inc. and Oxford Finance LLC, as Lender, dated February 16, 2018. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on February 18, 2018 (File No. 000-21429) and incorporated herein by reference.
10.33+	License Agreement, dated February 2, 2018, by and among ArQule, Inc., Sinovant Sciences Ltd. and Roivant Sciences Ltd. Filed as Exhibit 10.35 to the Company's Annual Report on Form 10-K filed on March 5, 2018 (File No. 000-21429) and incorporated herein by reference.
10.34+	License Agreement by and between the Company and Basilea Pharmaceutica International Limited, dated April 16, 2018, filed herewith. Filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on August 1, 2018 (File No. 000-21429) and incorporated herein by reference.
23.1	Consent of PricewaterhouseCoopers LLP, an Independent Registered Public Accounting Firm, filed herewith.
31.1	Rule 13a-14(a) Certificate of Chief Executive Officer, filed herewith.
31.2	Rule 13a-14(a) Certificate of Principal Financial Officer, filed herewith.
32	Rule 13a-14(b) Certificate of Chief Executive Officer and Principal Financial Officer, filed herewith.
101	The following materials from ArQule, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2018, formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets, (ii) Statements of Operations and Comprehensive Loss, (iii) Statements of Stockholders' Equity (Deficit) and Comprehensive Loss, (iv) Statements of Cash Flows, and (v) Notes to Financial Statements.

*
Indicates a management contract or compensatory plan.

+
Certain confidential material contained in the document has been omitted and filed separately with the Securities and Exchange Commission pursuant to Rule 406 of the Securities Act of 1933, as amended or Rule 24b-2 of the Securities and Exchange Act of 1934, as amended.

ITEM 16. FORM 10-K SUMMARY
The optional summary in Item 16 has not been included in this Form 10-K.
89

TABLE OF CONTENTS

SIGNATURES

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

ArQule, Inc.

By: /s/ Paolo Pucci

Paolo Pucci

Chief Executive Officer

Date: March 7, 2019

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

SIGNATURE	TITLE	DATE
/s/ Paolo Pucci Paolo Pucci	Chief Executive Officer and Director (Principal Executive Officer)	March 7, 2019
/s/ Peter S. Lawrence Peter S. Lawrence	President and Chief Operating Officer (Principal Financial Officer)	March 7, 2019
/s/ Robert J. Weiskopf Robert J. Weiskopf	Chief Financial Officer and Treasurer (Principal Accounting Officer)	March 7, 2019
/s/ Patrick J. Zenner Patrick J. Zenner	Director—Chairman of the Board	March 7, 2019
/s/ Timothy C. Barabe Timothy C. Barabe	Director	March 7, 2019
/s/ Susan L. Kelley Susan L. Kelley	Director	March 7, 2019
/s/ Ronald M. Lindsay Ronald M. Lindsay	Director	March 7, 2019
/s/ Michael D. Loberg Michael D. Loberg	Director	March 7, 2019
/s/ William G. Messenger William G. Messenger	Director	March 7, 2019
/s/ Ran Nussbaum Ran Nussbaum	Director	March 7, 2019