ARQULE INC
Form 10-K
March 05, 2018
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-K
ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF
THE SECURITIES EXCHANGE ACT OF 1934
FOR THE FISCAL YEAR ENDED DECEMBER 31, 2017 COMMISSION FILE NUMBER: 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 and posted on its Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405) of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No
Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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. (Check one):

Large accelerated filer	Accelerated filer
Non-accelerated filer	Smaller reporting company
(Do not check if a 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, 2017 was: $88,251,483.

There were 87,110,202 shares of the registrant's common stock outstanding as of February 20, 2018.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the definitive proxy statement for the Registrant's Annual Meeting of Stockholders to be held on May 8, 2018 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, 2017, are incorporated by reference into Part III of the Form 10-K.

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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 thereof, 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 drug candidates, are delayed or suspended, if our compounds 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.

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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 five 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 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 drugs, we intend 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 drugs if approved. The pipeline includes the following wholly-owned compounds:

- ARQ 531, an orally bioavailable, potent and reversible inhibitor of both wild type and C481S-mutant BTK, in Phase 1 for B-cell malignancies refractory to other therapeutic options;

- Miransertib (ARQ 092), a selective inhibitor of AKT, a serine/threonine kinase, in Phase 1/2 in rare Overgrowth Diseases and in Phase 1 in the rare disease, Proteus syndrome, in partnership with the National Institutes of Health (NIH); also in Phase 1b in oncology in combination with the hormonal therapy, anastrozole;

- Derazantinib (ARQ 087), a multi-kinase inhibitor designed to preferentially inhibit the FGFR family of kinases, in a registrational trial in intrahepatic cholangiocarcinoma (iCCA) in patients with FGFR2 fusions;

- ARQ 751, a next-generation inhibitor of AKT, in Phase 1 for solid tumors harboring the AKT1 or PI3K mutation; and

- ARQ 761, a ß-lapachone analog being evaluated as a promoter of NQO1-mediated programmed cancer cell death, in Phase 1/2 in multiple oncology indications in partnership with The University of Texas Southwest Medical Center.

Tivantinib (ARQ 197), an orally administered, small molecule inhibitor of the c-Met receptor tyrosine kinase and its biological pathway is no longer being developed. We licensed commercial rights to tivantinib for human cancer indications to Daiichi Sankyo Co., Ltd. (“Daiichi Sankyo”) in the U.S., Europe, South America and the rest of the world, excluding Japan and certain other Asian countries, where we licensed commercial rights to Kyowa Hakko Kirin Co., Ltd. (“Kyowa Hakko Kirin”).

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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 principal and approval. Derazantinib (formerly, ARQ 087) is in a potential fast-to-market, registrational trial in approximately 100 patients with iCCA with FGFR2 fusions; ARQ 531 is in a Phase 1a trial and will be advanced into a Phase 1b study where it will have the opportunity to show rapid proof of principle in the biomarker–defined C481S-mutant BTK patient population; and miransertib (formerly, ARQ 092) is in a Phase lb trial in combination with the aromatase inhibitor, anastrozole, in patients with the AKT1 mutation that could quickly lead to a late-stage development strategy.

- 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 drug candidates are being developed in clinical trials with biomarker-defined populations.

- Expand into 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. In all instances, we pursue a biomarker-defined precision medicine strategy in areas of high unmet need that also provide the opportunity for accelerated development. We launched a Phase 1/2 trial in PIK3CA-Related Overgrowth Spectrum (PROS) in Q2 of 2017 and intend to launch a registrational program in Proteus syndrome with the NIH in 2018.

- 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 technology to identify patient subsets with the highest likelihood of clinical benefit. In later-stage clinical development, particularly in registrational trials such as our trial in iCCA with derazantinib, we collaborate with experienced diagnostic partners to support our clinical and commercial precision medicine strategy.

- Benefit from the resources and strength of 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.

- Focus on cancer, a market with large unmet need. Cancer is the second most common cause of death in the U.S. According to the American Cancer Society, in 2017 approximately 601,000 cancer-related deaths were projected to occur and more than 1.7 million new cases of cancer were projected to be diagnosed in the U.S. Demographic trends and improved screening are expected to increase the rate of cancer diagnoses, as approximately 87 percent of cancers occur in the over-50-year-old population. We launched two new trials in oncology in 2017, a registrational trial with our FGFR inhibitor, derazantinib (ARQ 087) in iCCA, and a Phase 1a/b study with our BTK inhibitor, ARQ 531, in B-cell malignancies.

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GOALS FOR PIPELINE DEVELOPMENT

The chart below summarizes the current stage of our proprietary pipeline of product candidates and our goals for the next stage of each candidate's development.

OUR PRODUCT CANDIDATES

BTK Program: ARQ 531

Overview

ARQ 531 is an investigational, orally bioavailable, potent and reversible inhibitor of both wild type and C481S-mutant Bruton's tyrosine kinase (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, somatic mutation (C481S) of the BTK 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 C481S-mutant BTK and in other indications where ibrutinib is not highly effective. The Company intends to pursue an expedited development strategy in patients with the C481S mutation and also explore other indications in B-cell malignancies where ARQ 531 shows greatest promise. The company filed an IND application in Q1 2017 and began dosing a Phase 1a/b clinical trial for ARQ 531 in Q3 2017.

Background on BTK Inhibitors

B-cell malignancies, like 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.

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Currently it is estimated that approximately 20% of patients treated with ibrutinib become refractory, or resistant, to ibrutinib, and this incidence rate 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 ibrutinib binding to BTK, thus potentially positioning ARQ 531 as a targeted therapy for CLL, DLBCL and MCL patients harboring C481S-mutant BTK who have developed resistance to 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 inhibitor of both wild type and C481S-mutant BTK
- Potently inhibits activation of the C481S mutant with long residence time
- Showed remarkable efficacy in in vivo TCL1 mouse model, improving survival to a greater extent than ibrutinib
- Good ADME profile and excellent oral bioavailability in several species
- Composition of matter patent protection through December 2035

Preclinical Development of ARQ 531

The first preclinical data on ARQ 531 were presented at the Pan Pacific Lymphoma Conference and the American Society of Hematology Conference in 2016.

At the Pan Pacific Lymphoma Conference data from the TMD8 xenograft mouse model demonstrated strong in vivo target and pathway inhibition of BTK by ARQ 531 with sustained tumor growth inhibition. ARQ 531 also demonstrated biochemical inhibition of both wild type and C481S-mutant BTK at nanomolar levels and potent cellular inhibition in C481S-mutant BTK cells that are resistant to ibrutinib. Data presented also demonstrated a distinct kinase selectivity profile of ARQ 531 with inhibitory activity against key oncogenic targets related to ibrutinib resistance. Additionally, the data showed that the compound potently suppressed cell proliferation of hematological malignancies in vitro, with B-cell receptor signaling inhibition. ARQ 531 also demonstrated strong in vivo target and pathway inhibition of pBTK with potent and durable growth suppression.

At the 2016 American Society of Hematology Annual Meeting in a poster presented by our collaborator, The Ohio State University, multi-targeted inhibition of cytokine, chemokine, and BCR pathways by ARQ 531 decreased activation, migration, and viability of CLL cells. Additional data presented showed that unlike ibrutinib, ARQ 531 inhibits activation of C481S-mutated BTK variants and maintains cytotoxicity in ibrutinib resistant clones. The molecule was also shown to demonstrate remarkable efficacy in an in vivo TCL1 mouse model, improving survival to a greater extent than ibrutinib and restoring granulocyte production. Our collaborator concluded these data warrant advancing ARQ 531 into clinical trials.

Clinical Development of ARQ 531

We filed a U.S. IND application for ARQ 531 in Q1 of 2017 and commenced a Phase 1a/b trial in Q3 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. We are currently dosing patients in the Phase 1a portion of

the trial and intend to advance ARQ 531 into a Phase 1b basket study of approximately 80 – 100 patients, including those with the C481S mutation and other B-cell

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malignancies where ARQ 531 is expected to show promise in late 2018 or early 2019. 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.

AKT Program: Miransertib (ARQ 092) and ARQ 751

Overview

Miransertib (ARQ 092) and 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.

Dysregulation of AKT is also a driver of certain rare proliferative disorders. For example, the E17K mutation of AKT1 causes Proteus syndrome, a rare non-cancerous segmental overgrowth disorder, and the analogous PIK3CA-Related Overgrowth Spectrum (PROS) is caused by genetic alterations in the PI3K pathway. 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 drug candidates in various stages of clinical testing in oncology. Miransertib and ARQ 751 are investigational, allosteric inhibitors of AKT1, 2 and 3. Both molecules are derived from a proprietary chemical class with distinct ADME and pharmacokinetic (PK) properties. Because ARQ 092 and ARQ 751 do not bind AKT at the ATP binding site, they are differentiated from ATP-competitive inhibitors of AKT, and we believe may provide greater selectivity and possibly fewer toxicities.

Key Characteristics of Miransertib

- Potent, selective, allosteric pan-AKT inhibitor
- Clinical effect observed in a number of oncology patients showing RECIST responses
- Knock down of AKT signaling observed in patients at low doses, presenting attractive profile for treatment of rare diseases
- Manageable safety profile at higher doses in oncology
- Good drug-like properties

Preclinical Development of Miransertib

Preclinical studies of miransertib showed high potency against AKT1, 2 and 3, with nanomolar inhibition of downstream proteins. Cancer cells that harbor the E17K-AKT1 mutation, H1047R-PIK3CA mutation or are PTEN-null are the most sensitive to miransertib. Good tumor growth inhibition was observed in human tumor xenograft mouse models or 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)

We are collaborating with the National Institutes of Health (NIH) on the clinical development of miransertib for treatment of patients with Proteus syndrome. Proteus syndrome is driven by the E17K-AKT1 mutation, and data from the NIH's Phase 1 trial with miransertib confirmed biological

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activity in 5 out of 6 patients treated where the protocol defined decrease in AKT signaling of greater than 50% at the starting dose of 10 mg was observed. Having achieved in vitro and in vivo proof of concept, and in order to accelerate the development process, we initiated an ArQule-sponsored trial in Q2 2017 in patients with Overgrowth Diseases, including patients with Proteus syndrome, called PROS (PIK3CA-Related Overgrowth Spectrum) that we believe are sensitive to miransertib.

In our Phase 1/2 study in PROS, we are treating patients with extensive disease with the objective of identifying an effective dose as well as relevant potential endpoints for the next stage of clinical testing. In addition, we are providing, in selected cases, miransertib on a named patient basis. Based on requests received to date, these named patients are often younger, have rapidly progressing disease and are in significant need of potential treatment options. In addition, we plan to initiate with the NIH a registrational program with miransertib in patients with Proteus Syndrome in 2018.

Rare Pediatric Disease Designation

In Q4 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, 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.

Clinical Development of Miransertib – Oncology

Miransertib is being tested in two Phase 1 clinical trials for oncology indications. The first trial evaluates the safety, tolerability, PK and pharmacodynamics (PD) of miransertib patients with advanced solid tumors and recurrent malignant lymphomas to define a RP2D. The study is active and recruitment has been completed. In this study, miransertib demonstrated a manageable safety profile and showed single agent activity, achieving partial responses in a number of tumors harboring AKT1 or PI3K mutations. A Phase 1b 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 pharmacokinetic profile from miransertib may lead to more favorable dosing characteristics
- Prolonged growth inhibition in several tumor xenograft mouse models
- Good drug-like properties

Development of ARQ 751

ARQ 751 is our next generation AKT inhibitor. It is a highly potent pan-AKT inhibitor. Its profile is similar to miransertib but shows a 5 to15 fold increase in potency across binding, biochemical and cellular assays. ARQ 751 is differentiated at the ADME and PK levels, as it has lower volume of distribution and does not accumulate in tissues, making dosing ARQ 751 more precise. In GLP-toxicity studies in rats and monkeys, ARQ 751 had fewer and less severe rashes, a toxicity common to all AKT inhibitors in development.

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ARQ 751 is currently being tested in a Phase 1 clinical trial for oncology indications. The ongoing trial is a dose escalation study of ARQ 751 in patients with advanced solid tumors with AKT1, 2 and 3 genetic alterations, activating PI3K mutations or PTEN-null to define a RP2D.

FGFR Program: Derazantinib (ARQ 087)

Overview

Derazantinib (ARQ 087) is an investigational, 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. Current scientific literature suggests FGFR dysregulation exists in anywhere from 5% to 40% of these cancers.

Derazantinib has demonstrated in vivo inhibition of tumor growth and downstream signaling in tumors whose growth is driven by FGFR targets. Additionally, derazantinib has demonstrated favorable clinical data in a biomarker driven Phase 1/2 trial in iCCA targeting patients with FGFR2 fusions. Both the FDA and European Medicines Agency (EMA) have granted us orphan drug designation for this disease. We initiated a registrational, biomarker-driven trial in this indication in Q3 2017.

Background on FGFR Inhibitors

Increased understanding of tumor biology has led to the identification of tumor drivers like epidermal growth factor receptor, vascular endothelial growth factor and FGFR whose inhibition by targeted agents has shown antitumor activity and in some cases led to approval of drugs such as Tarceva™ and Nexavar™. The FGFR family consists of four genes encoding tyrosine kinase receptors (FGFR1, FGFR2, FGFR3, and FGFR4).

In human cancers, FGFRs have been found to be dysregulated by multiple mechanisms, including aberrant expression, mutations, chromosomal rearrangements, and amplifications. derazantinib is a potent FGFR inhibitor that shows strong anti-proliferative activity in cell lines harboring FGFR2 alterations. In clinical testing the molecule has demonstrated activity in cancerous tumors harboring FGFR2 fusions in iCCA and bladder cancers. Given the high unmet need and activity observed to date in our Phase 1/2 clinical trial, we have selected iCCA as our first indication in a registrational trial.

About iCCA

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, but treatment is based on the patient’s stage of the cancer when diagnosed and include resection, chemoradiation and systemic chemotherapy.

We are pursuing a precision medicine approach to iCCA because molecular characterization of iCCA by next generation sequencing (NGS) and fluorescence in situ hybridization (FISH) have enabled identification of genetic alterations that can potentially be treated by targeted therapies like derazantinib. Scientific studies suggest that 10% to 20% of the iCCA population has a FGFR2 fusion.

Key Characteristics of Derazantinib

- Multi-kinase inhibitor that potently inhibits FGFR1, 2 and 3 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

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Preclinical Development of Derazantinib

The first preclinical data on derazantinib was presented at a scientific congress in 2013, and in 2016 a more comprehensive drug profile was published in the journal, PLOS ONE. The data demonstrate potent in vitro and in vivo activity in FGFR2 driven models. Additionally, the data show derazantinib inhibits the active and inactive forms of FGFR1 and FGFR2 and has strong anti-proliferative activity in cell lines driven by FGFR2, inducing G1 cell cycle arrest and cell death in FGFR2 amplified cell lines. Further preclinical studies have demonstrated that biochemically, derazantinib potently inhibits FGFR1, FGFR2, mutant FGFR2 (N549H), and FGFR3 kinases, with IC50 values in the low nanomolar range in biochemical assays. Cell proliferation studies demonstrated that derazantinib has anti-proliferative activity in different cell lines with higher activity in those cells driven by FGFR dysregulation, including amplifications, fusions, and mutations.

Clinical Development of Derazantinib

ArQule began a Phase 1a trial with derazantinib for the treatment of advanced solid tumors in December 2012. The primary objective of the Phase 1 trial was to determine safety, tolerability and recommended Phase 2 dose (RP2D). In the Phase 1a study derazantinib showed a manageable safety profile in subjects with advanced solid tumors. The RP2D has been defined as 300 mg once-a-day.

The Phase 1a trial enrolled 12 iCCA patients including five with FGFR2 fusions, six without FGFR2 fusions and one unknown mutation. Of the five iCCA patients with FGFR2 fusions, two reported partial responses as a best response with tumor shrinkage of over 30%, and three reported stable disease as a best response. Of the six iCCA patients without FGFR2 fusions, all progressed while on therapy.

Based on clinical data derived from the Phase 1a trial, we decided to initiate a Phase 1b trial for ARQ 087 in solid tumors with FGFR2 fusions and in parallel, we initiated the Phase 2 portion of the trial to enroll second-line iCCA patients with FGFR2 fusions.

In total 29 iCCA second-line patients with FGFR2 fusions were enrolled in the Phase 1a and Phase 2 portions of the trial. We observed six partial responses out of 29 evaluable patients representing a 21% response rate. A retrospective analysis of the current literature suggests a response rate of approximately 7.7% to chemotherapy in the second-line iCCA population. Additionally, a 83% disease control rate and median time on treatment of over 26 weeks were observed in the trial, and the drug has demonstrated a manageable side effect profile.

The Company initiated a registrational, biomarker-driven trial in second-line iCCA patients with FGFR2 fusions in Q4 2017. The trial is designed to be single-arm, response rate driven and will enroll approximately 100 patients in the U.S. and EU. If the trial is successful, the Company will seek conditional approval of derazantinib with the FDA.

NQO1 Program: Cancer

We are collaborating with the University of Texas Southwestern Medical Center on the clinical development of ARQ 761, an intravenously administered analogue of ß-lapachone, a naturally occurring substance. ARQ 761 is a pro-drug of ARQ 501, which has demonstrated in vitro activity against a wide range of solid tumors. Phase 1a testing with ARQ 761 identified anti-cancer activity as measured by tumor responses that occurred exclusively in a portion of the patient population with high levels of NQO1, the mechanistic target of the compound. Consequently, Phase 1b expansion cohorts for ARQ 761 will focus on patients whose tumors have high levels of NQO1. In 2015, a Phase 1b/2 trial was initiated with ARQ 761 in pancreatic cancer.

CORPORATE PARTNERSHIPS

Daiichi Sankyo Co., Ltd.

As previously reported, on December 18, 2008, we entered into a license, co-development and co-commercialization agreement with Daiichi Sankyo to conduct research, clinical trials and the commercialization of tivantinib in human cancer indications in the U.S., Europe, South America and the

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rest of the world, excluding Japan, China (including Hong Kong), South Korea and Taiwan, where Kyowa Hakko Kirin has exclusive rights for development and commercialization. On February 17, 2017, we and Daiichi Sankyo announced that the MET-IV-HCC trial did not meet its primary end point of improving OS. As a result, Daiichi Sankyo and we have discontinued development of tivantinib.

Kyowa Hakko Kirin Co., Ltd

As previously reported, on April 27, 2007, we entered into an exclusive license agreement with Kyowa Hakko Kirin to develop and commercialize tivantinib in Japan and parts of Asia. Revenue for this agreement was recognized using the contingency-adjusted performance model with an estimated development period through December 31, 2016. On March 27, 2017, we reported that Kyowa Hakko Kirin announced top-line results of the JET-HCC Phase 3 trial of tivantinib in Japan, and that the trial did not meet its primary endpoint of improving PFS. As a result, Kyowa Hakko Kirin has discontinued development of tivantinib in the Asian territory.

Roivant Sciences

In February 2018 Roivant Sciences Ltd. and ArQule, Inc. announced the initiation of a collaboration to pursue the development of derazantinib, a pan-FGFR (fibroblast growth factor receptor) inhibitor, in Greater China. As part of the collaboration, ArQule has granted a Roivant subsidiary (Sinovant) an exclusive license to develop and commercialize derazantinib in the People's Republic of China, Hong Kong, Macau, and Taiwan. Deal terms include an upfront payment to ArQule of $3 million and an additional $2.5 million development milestone within the first year. ArQule is also eligible for an additional $82 million in regulatory and sales milestones. Upon commercialization, ArQule will receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in the Greater China territory.

PATENTS AND PROPRIETARY RIGHTS

We rely principally on patent and trade secret protection for our intellectual property, both in the U.S. and other countries. While many patent applications have been filed in the U.S., the European Union ("E.U.") and other foreign countries with respect to our drug 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.

With respect to our AKT and FGFR programs, we have issued patents and pending patent applications in the U.S., the E.U. and other foreign jurisdictions. For our AKT program, we have four issued patents in the U.S. covering the composition of matter for our lead AKT compounds. The expiration dates of these patents range from December 2031 to June 2032. We also have granted patents in the E.U., Australia, the People's Republic of China, Hong Kong, Israel, Japan, South Korea, Mexico, Malaysia, Macau, New Zealand, the Philippines, the Republic of Singapore, Russia, Taiwan and South Africa. We understand that these patents will expire between December 2030 and June 2032. Furthermore, we have

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three issued patents in the U.S. relating to the synthesis and polymorphs of our lead AKT compounds. The expiration dates of these patents range from March 2035 to April 2035. For our FGFR program, we have one issued patent in the U.S. covering the composition of matter for our lead FGFR compounds. This patent will be adjusted beyond its normal expiration date of December 2029 to January 2031. We also have granted patents in the E.U., Australia, the People's Republic of China, Israel, Japan, South Korea, Taiwan, the Philippines, Mexico, Macau and South Africa. We understand that 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.

ARQ 761 is being investigated as a potential NQO1 inhibitor. We have an issued patent in the U.S. covering the composition of matter of this compound, pharmaceutical compositions containing this compound, and the therapeutic uses of this compound in the treatment of cancer. The U.S. Patent and Trademark Office has determined that the term of the patent will be adjusted beyond its normal expiration date of April 2028 to December 2028. We also have issued patents in the E.U., Australia, the People's Republic of China, Canada, Hong Kong, Mexico and Taiwan covering the composition of matter of this compound. We understand that these patents will expire in April 2028.

For our BTK program, we have issued patents and pending patent applications in the U.S. and other 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 the patent is December 23, 2035. We have also filed patent applications in the U.S. and foreign jurisdictions covering other analogs of ARQ 531. If these applications are issued as patents, they will expire in August 2037.

For Tivantinib, we have maintained all issued patents covering composition of matter, pharmaceutical composition, methods of use, formulation, manufacturing, combination with other anticancer agents in the U.S. and other foreign territories.

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, biotechnology 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 drug 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 drug 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., Ariad Pharmaceuticals, Inc., Astellas Pharma, Inc., Array BioPharma Inc., AstraZeneca PLC, Celgene Corporation, Curis, Inc., 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 087, we are aware of a number of companies that are or may be pursuing a number of different approaches to FGFR inhibition, including Ariad Pharmaceuticals, 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

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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 Meyers Squibb, Cellact Pharma Gmbh, Concordia Healthcare, Dainippon Sumitomo Pharma Co., Ltd., Delcath Systems, Inc., Exelixis, Novartis, Oncotherapy Services, Inc. and Spectrum Pharmaceuticals, Inc.

Regarding ARQ 092, 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 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., LOXO Oncology, Roche and Sunesis Pharmaceuticals. Moreover, numerous companies are also pursuing inhibitors of wild-type BTK, including AbbVie with its drug, IMBRUVICA™, and Astra Zeneca 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™.

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 U.S. 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.

Preclinical and Clinical Studies

Generally, in order to gain marketing authorization, a company first must conduct preclinical studies in the laboratory and in animal models to gain preliminary information on a compound's activity and to identify potential safety problems. Preclinical studies must be conducted in accordance with applicable regulations of the relevant regulatory authority (e.g. FDA in the U.S., European Medicines Agency ("EMA") in E.U.). The results of these studies are submitted as a part of an IND application with the FDA or a Clinical Trial Application ("CTA") application with the appropriate regulatory authority outside of the U.S. The regulatory agency involved must review the data in the application before human clinical trials of an investigational drug can commence. If the regulatory authority does not object, a drug developer can begin clinical trials after expiration of a specified statutory period following submission of the application. Notwithstanding that the regulatory authority did not object during the applicable post-submission review period, the regulatory authority may at any time re-evaluate the adequacy of the application and require additional information about any aspect of the IND or CTA application and corresponding clinical trial, e.g. preclinical testing, drug formulation and manufacture, dosing regimens and drug administration or potential safety risks.

In order to eventually commercialize any products, we or our collaborator will be required to initiate and oversee clinical studies under an IND or CTA to demonstrate the safety and efficacy that are necessary to obtain marketing approval. Clinical trials are normally done in three phases and generally take

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several years, but may take longer to complete. Furthermore, a regulatory authority may suspend clinical trials at any time if it believes that the subjects participating in trials are being exposed to unacceptable risks or if the regulatory authority finds deficiencies in the conduct of the trials or other problems with our product under development.

In addition, information about and results from any clinical studies we conduct may be subject to public disclosure (on www.clinicaltrials.gov). A rule that went into effect on January 18, 2017 broadens the clinical trial submission requirements to apply to results of information for unapproved drugs, regardless of whether FDA approval is being sought, unless a waiver is granted. Prior to enactment of the rule, disclosure of results for trials of unapproved drugs could be delayed until FDA approval, but the new rule generally limits the allowable delay period for such results.

Companion Diagnostic Development and Approval

In addition to these requirements, with some clinical candidates for which there is a valid predictive biomarker, a diagnostic test known as a companion diagnostic ("CDx") 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. In the U.S., such companion diagnostics are regulated as medical devices, and marketing authorization is usually based on approval of a Premarket Approval ("PMA") application which establishes the predictive value of the test in the context of a registration trial of the drug; this application is submitted in the U.S. to FDA's Center for Devices and Radiological Health. Approval of a PMA for a companion diagnostic for a clinical candidate is not guaranteed, and requires a determination by FDA that the PMA contains sufficient valid scientific evidence to assure that the diagnostic is safe and effective for its intended use. In the European Economic Area ("EAA") approval is achieved by obtaining a "CE mark" by submitting a Declaration of Conformity under the Medical Device Directive.

Marketing Approval Process

After completion of clinical trials of a new product, regulatory marketing approval must be obtained. If the product is classified as a new pharmaceutical, we or our collaborators will be required to file a New Drug Application ("NDA") or Marketing Authorization Application ("MAA"), and receive approval before commercial marketing of the drug. The marketing application contains, among other things, the results of the non-clinical and clinical testing of the drug. Marketing applications submitted to any regulatory authority can take several years to obtain approval and the regulatory authority is not obligated to grant approval at all. A regulatory agency can condition marketing approval on the conduct of costly post-marketing follow-up studies or can place restrictions on the sale or marketing of the drug in order to manage risks.

In the U.S., once the FDA receives an NDA submission, the agency has 60 days to determine whether to receive the application for filing. Once accepted for filing, FDA conducts an in depth review in accordance with performance goals timelines to which the agency has agreed. Most NDAs are generally reviewed within ten to twelve months, unless the application qualifies for and the sponsor obtains a priority review designation, in which case the review period is generally six to eight months. These review periods can be extended by three months in order for FDA to consider additional information. Various programs are available that are intended to facilitate the development of and/or expedite the review of new drugs intended to address unmet medical need in the treatment of serious or life-threatening conditions provided certain specified conditions are met. Such programs include priority review designation, fast track designation, breakthrough therapy designation, and the accelerated approval program.

In making an approval determination for an NDA, FDA may elect to convene an advisory committee to provide independent advice and recommendations to FDA related to approval of the application. Approval of an NDA may also be conditioned upon the manufacturing facility at which the drug will be manufactured successfully completing an FDA pre-approval inspection (PAI). In addition, once NDA-approval is obtained, if certain changes are made to a drug, including to its manufacturing or its labeling, the changes could require prior FDA approval through an NDA supplement, and, depending on the specific change, could require the submission of new clinical data.

Upon approval, a new drug may be eligible for one or more periods of exclusivity that can delay the submission or approval of certain marketing applications. In the U.S. the Federal Food, Drug, and

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Cosmetic Act ("FDCA") as amended by the Hatch-Waxman Act, provides a 5-year period of non-patent market exclusivity to the first sponsor to obtain approval of a new chemical entity (referred to as "NCE exclusivity"). During a drug's 5-year NCE exclusivity period, FDA may not accept for review an abbreviated new drug application ("ANDA") or a section 505(b)(2) application submitted by another company that references the protected application unless the sponsor of the new drug has a right of reference to the data required for approval. An exception exists when an ANDA or 505(b)(2) application contains a patent invalidity or non-infringement certification, in which case FDA may accept the application for review after 4 years rather than after 5 years. For drugs that do not qualify for NCE exclusivity, the Hatch-Waxman Act also provides for a 3-year exclusivity period for NDAs, including 505(b)(2) NDAs, and supplements to NDAs where approval of the NDA requires new clinical investigations conducted or sponsored by the applicant that are deemed by FDA to be essential to approval of the application (e.g., approval for a new indication). Such 3-year exclusivity bars FDA from approving any ANDA or 505(b)(2) application that relies on the information supporting the approval of the drug or the change to the drug for which information was submitted and exclusivity granted.

Neither Hatch-Waxman 5-year NCE exclusivity or 3-year exclusivity blocks the submission or approval of a full NDA that does not reference or rely on a previously approved application even if the drug that is the subject of the new NDA is considered to be the same chemical entity as the previously approved drug with unexpired 5 or 3-year exclusivity. In contrast, a type of exclusivity that blocks approval of subsequent applications irrespective of referencing is orphan drug exclusivity. Under the FDCA, as amended by the Orphan Drug Act of 1983, FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition, which is defined generally as a disease or condition that affects fewer than 200,000 individuals in the United States. If an orphan-designated drug is the first such drug to obtain market approval for its orphan designated indication, the drug may receive a 7-year period of orphan drug exclusivity. Orphan drug exclusivity blocks the approval of any marketing application for a drug that is considered the same drug as the orphan-protected drug for the same orphan-protected indication. If a subsequent drug is considered the same drug as a protected orphan drug, in order for the sponsor of the subsequent drug to be able to obtain marketing approval for the same protected orphan indication during that exclusivity period, the sponsor must demonstrate that its drug is clinically superior to the previously approved drug with unexpired orphan exclusivity. If a previously approved same drug does not have unexpired orphan exclusivity, while the sponsor of a subsequent orphan-designated same drug in that scenario would not be required to demonstrate superiority over the previously approved drug in order to obtain marketing approval, a demonstration of superiority would be required in order for the subsequent drug to qualify for its own period of 7-year orphan exclusivity.

Our proprietary pipeline of product candidates is being developed in targeted, biomarker-defined patient populations. By seeking out subgroups of patients that are most likely to respond to our drugs, we intend to identify small, often orphan, indications that allow for focused and efficient development. In particular, our product candidate derazantinib has been granted orphan designation for iCCA and miransertib has received such designation for Proteus syndrome. Consequently, if derazantinib or miransertib is the first such drug to obtain marketing approval for iCCA or Proteus syndrome, respectively, then it could qualify for a 7-year period of orphan drug exclusivity. If, however, another sponsor's product that is considered the same drug as either derazantinib or miransertib is first to market and obtains orphan exclusivity for the applicable indication, the exclusivity could block the approval of derazantinib or miransertib, as the case may be, absent a demonstration of clinical superiority.

Fast Track Designation and Accelerated Approval

The FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the Fast Track program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a Fast Track drug concurrent with, or after, the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for Fast Track Designation within 60 days of receipt of the sponsor's request.

Under the Fast Track program and the FDA's Accelerated Approval regulations, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over

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existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of post-approval clinical trials, often referred to as Phase 4 trials, to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to priority review by the FDA.

If a submission is granted Fast Track Designation, the sponsor may engage in more frequent interactions with the FDA, and the FDA may review sections of the NDA before the application is complete. This rolling review is available if the applicant provides, and the FDA approves, a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, the FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, Fast Track Designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Breakthrough Therapy Designation

The FDA is also required to expedite the development and review of the application for approval of drugs that are intended to treat a serious or life-threatening disease or condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Under the Breakthrough Therapy program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a breakthrough therapy concurrent with, or after, the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for Breakthrough Therapy designation within 60 days of receipt of the sponsor's request.

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

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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.

Postmarketing and Other Requirements

Even if regulatory clearances are obtained, a marketed product is subject to continual review and ongoing regulatory obligations. If and when a regulatory authority approves any of our or our collaborators' products under development, the manufacture and marketing of these products will be subject to continuing regulation, including compliance with current Good Manufacturing Practices ("cGMP"), adverse event reporting requirements and prohibitions on promoting a product for unapproved uses or making false or misleading statements or omissions with respect to a drug in advertising or promotion. Later discovery of previously unknown problems or failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Various federal and, in some cases, state statutes and regulations also govern or influence the manufacturing, safety, labeling, storage, record keeping and marketing of pharmaceutical products.

For marketing outside the U.S., we or our partners will be subject to foreign regulatory requirements governing human clinical trials, marketing approval and post-marketing activities for pharmaceutical products and biologics. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary widely from country to country.

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:

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

- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third party payers that are false or fraudulent;

- federal transparency laws, including the federal Physician Payment Sunshine Act, that requires drug manufacturers to disclose payments and other transfers of value provided to physicians and teaching hospitals;

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

- provisions of HIPAA, as amended by the Health Information Technology and Clinical Health Act and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information;

- state law equivalents of each of the above federal laws; and

- state price transparency laws.

EMPLOYEES

As of December 31, 2017, we employed 32 people in Burlington, Massachusetts. Of that total, 18 are engaged in research and development and 14 in general and administration, and 9 hold PhDs, 4 hold MDs and 6 hold Masters Degrees in the sciences.

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CERTAIN OTHER INFORMATION

We file annual, quarterly and current reports, proxy statements and other information with the Securities and Exchange Commission ("SEC"). You may read and copy any document we file at the SEC's Public Reference Room at 100 F Street, N.E., Washington D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the public reference room. 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. The Company's 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 the Company's website.

EXECUTIVE OFFICERS

Set forth below is certain information regarding our current executive officers, including their respective ages as of February 1, 2018.

NAME	AGE	POSITION
Paolo Pucci	56	Chief Executive Officer and a Director
Peter S. Lawrence	54	President and Chief Operating Officer
Robert J. Weiskopf	67	Chief Financial Officer and Treasurer
Dr. Brian Schwartz	56	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

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and structuring, syndication and business expansion activities. Previously, Mr. Lawrence was an attorney and partner at Mintz, Levin, Cohn, Ferris Glovsky and Popeo, P.C., from 1991 to 2001. 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 through periods of rapid growth and transformative corporate events. His public financing experiences include the initial public offering and numerous financings for America Online Inc. (AOL), as well as public financings for Biogen, Human Genome Sciences, Hybridon and many other companies. He worked on numerous mergers and acquisitions, including Roche/Compuchem, AOL/Time Warner, Steinway Piano, DEC/Intel, and Mitotix/GPC Biotech. Mr. Lawrence worked at Gaston & Snow from 1989 to 1991 in the firm's Corporate Law Department. He holds a Bachelor's degree from Amherst College and a J.D. from Boston University School of Law.

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 OUR INDUSTRY AND BUSINESS STRATEGY

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. 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. Our viability as a company may depend, in part, on our ability to continue to create product candidates for ourselves and our collaborators. Numerous significant factors will affect the success of our drug research and development efforts, including the biology and chemistry complexity

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involved, availability of appropriate technologies, the uncertainty of the scientific process and the capabilities and performance of our employees. Our research and development capabilities may not be adequate to develop additional, viable drug candidates.

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 products, 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.

Clinical trials must meet FDA and foreign regulatory requirements. We have limited experience in designing, conducting and managing the preclinical studies and clinical trials necessary to obtain regulatory approval for our product candidates in any country. 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. These problems could include our inability to manufacture or obtain sufficient quantities of materials produced in accordance with current Good Manufacturing Practice, or cGMP, for use in our clinical trials, conduct clinical trials at our preferred sites, enroll a sufficient number of patients for our clinical trials at one or more sites, or begin or successfully complete clinical trials in a timely fashion, if at all. Furthermore, we, the FDA or foreign regulatory agencies may suspend clinical trials of our product candidates at any time if we or they believe the subjects participating in the trials are being exposed to unacceptable health risks as a result of adverse events occurring in our trials or if we or they find deficiencies in the clinical trial process or conduct of the investigation.

Acceptable 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 HCC did not lead to a positive outcome in our Phase 3 trials in HCC.

Although it is part of our strategy to pursue clinical development to take advantage of available accelerated regulatory approval processes, there is no guarantee that our product candidates will show the evidence predictive of clinical benefit necessary to qualify for such regulatory treatment.

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.

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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. 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:

- 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 clinical research organizations, or CROs, clinical investigators and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different 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;

- trial results may not meet the level of statistical significance required by the FDA or other regulatory agencies;

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

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

- 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

- the FDA or other regulatory agencies may lack experience in evaluating the safety and efficacy of our drugs, which could lengthen the regulatory review process.

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.

Clinical trials may also be delayed or repeated as a result of ambiguous or negative interim results or unforeseen complications in testing. In addition, a clinical trial may be suspended or terminated by us, the FDA, the institutional review board ("IRB") overseeing the clinical trial at issue, any of our clinical trial sites with respect to that site, or other regulatory authorities due to a number of factors, including:

- failure to design appropriate clinical trial protocols;

- failure by us, our employees, our CROs or their employees to conduct the clinical trial in accordance with all applicable FDA, Drug Enforcement Administration ("DEA") or other regulatory requirements or our clinical protocols;

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- inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities resulting in the imposition of a clinical hold;

- discovery of serious or unexpected toxicities or side effects experienced by study participants or other unforeseen safety issues;

- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional trials and studies and increased expenses associated with the services of our CROs and other third parties; lack of effectiveness of any product candidate during clinical trials;

- slower than expected rates of subject recruitment and enrollment rates in clinical trials;

- 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;

- inability or unwillingness of medical investigators to follow our clinical protocols; and

- unfavorable results from on-going clinical trials and preclinical studies.

Additionally, changes in applicable regulatory requirements and guidance may occur, and we may need to amend clinical trial protocols to reflect these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for reexamination, which may impact the costs, timing or successful completion of a clinical trial. 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.

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 METIV-HCC trial. 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.

We depend substantially on the successful completion of Phase 3 or other pivotal clinical trials for our product candidates. The positive clinical results obtained for our product candidates in Phase 2 clinical studies may not be repeated in Phase 3.

We have experienced significant delays and obstacles in a number of clinical trials. Our product candidates are subject to the risks of failure inherent in pharmaceutical development. Before obtaining regulatory approval for the commercial sale of any product candidate, we and, as applicable, our collaborators must successfully complete Phase 3 or other pivotal clinical trials. Negative or inconclusive results of a Phase 3 or other pivotal clinical study

could cause the FDA to require that we repeat it or conduct additional clinical studies or abandon further development of an indication or drug candidate.

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 drug 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

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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 generate revenues, cause us to incur additional expense and cause the market price of our common stock to decline significantly.

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, 2017 we have incurred cumulative losses of approximately $534 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 drug 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.

On January 6, 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. 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:

- sell, transfer or otherwise dispose of any of our business or property, subject to limited exceptions;
- make material changes to our business or management;
- 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;

- enter into transactions with our affiliates;
- repay subordinated indebtedness;
- enter into certain exclusive licensing arrangements; or
- make certain investments.

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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 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, which could force us to delay, reduce or eliminate our drug discovery, product development and commercialization activities.

Volatility and disruption in the global capital and credit markets in recent years have led to a tightening of business credit and investment capital in the U.S. and internationally. If global economic and financial market conditions deteriorate or remain weak for an extended period of time, our efforts to raise capital will face additional difficulties. Developing drugs, conducting clinical trials, and commercializing products are expensive. 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 and our ability to share such costs of our clinical development efforts with third parties;

- 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;

- the costs and timing of commercializing our product candidates, including establishing or contracting for sales, marketing and distribution capabilities, if any such 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 losses ("NOL") 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, 2017, we had federal NOL, state NOL, and research and development credit carryforwards of approximately $409 million, $228 million and $28 million respectively, which expire at

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various dates through 2037. 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 NOL and research and development credit carryforwards through January 31, 2018 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 NOL 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.

RISKS RELATED TO REGULATORY APPROVAL

Our product candidates are subject to a lengthy and uncertain regulatory process that may not result in the necessary regulatory approvals, which would adversely affect our ability to commercialize products. We have only limited experience in regulatory affairs.

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 U.S. and by comparable authorities in other countries, for example EMA in the E.U. These regulations govern or influence the manufacturing, assessment of benefit and risk, safety, labeling, storage, commercialization and reimbursement of these products.

Failure to obtain regulatory approval for a product candidate would prevent us from commercializing that product candidate. We have not applied for or received regulatory approval to market any of our product candidates in any jurisdiction and have only limited experience in preparing and filing the applications necessary to gain regulatory approvals. The process of obtaining regulatory approvals is expensive, can take many years, if approval is obtained at all, and can vary substantially based upon the type, complexity and novelty of the product candidate involved.

The regulatory process requires preclinical testing, and data obtained from preclinical and clinical activities are susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. In addition, the results of later trials may not confirm the positive results of earlier preclinical studies or trials. Delays or rejections may also be encountered based upon changes in regulatory policy for product approval during the period of product development and regulatory agency review. Changes in regulatory approval policy, regulations or statutes or the process for regulatory review during the development or approval phases of our product candidates may cause delays in the approval or rejection of an application.

A company first must conduct preclinical studies in the laboratory and in animal models to gain preliminary information on a candidate compound’s activity and to identify potential safety problems. Preclinical studies must be conducted in accordance with applicable regulations of the relevant regulatory authority (e.g. the FDA in the U.S., the EMA in E.U.). The results of these studies are submitted as a part of an IND application with the FDA or a Clinical Trial Application (“CTA”) with the appropriate regulatory authority outside of the U.S. The regulatory agency involved must review the data in the application before human clinical trials of an investigational drug can commence. If the regulatory authority does not object, a drug developer can begin clinical trials after expiration of a specified statutory period following submission of the application. Notwithstanding that the regulatory authority does not respond during the thirty-day, post-submission review period, the regulatory authority may at any time re-evaluate the adequacy of the application and require additional information about any aspect of the IND or CTA and corresponding clinical trial, e.g. preclinical testing, drug formulation and manufacture, dosing regimens and drug administration or potential safety risk. Before a new marketing application can be filed with the FDA or other regulatory authority, the product candidate must undergo extensive clinical trials. Any clinical trial may fail to produce results satisfactory to the regulatory authority, typically for lack of ‘efficacy or for safety risks. For example, the regulatory authority could determine that the design of a clinical trial is inadequate to produce reliable results or convincing results.

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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. In collaboration with our partners and third party developers, we plan to develop companion diagnostics to help us to identify patients within a particular subset, both during our clinical trials and in connection with the 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 our products. 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 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, which could adversely affect and/or delay or prevent the development, market approval or commercialization of our product candidates. 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.

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 initial 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, 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.

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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.

We face potential liability related to the privacy of health information we obtain from research institutions.

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.

RISKS RELATED TO COLLABORATIONS

Part of our business strategy involves collaborative out-licensing of our pruduct candidates while retaining commercialization or co-promotional rights in parts of the world. 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. We may enter into these collaborations to obtain external financing for drug development and to obtain access to drug development and commercialization expertise. The availability of partners depends on the willingness of pharmaceutical and biotechnology companies to collaborate in drug discovery activities. Only a limited number of pharmaceutical and biotechnology companies would fit our requirements. The number could decline further through consolidation, or the number of collaborators with interest in our drugs could decline. If the number of our potential collaborators were to decline, the remaining collaborators may be able to negotiate terms less favorable to us.

We face significant competition in seeking drug development collaborations, both from other biotechnology companies and from the internal capabilities and drug pipelines of the pharmaceutical and biotechnology companies themselves. This competition is particularly intense in the oncology field. Our ability to interest such companies in forming collaborations with us will be influenced by, among other things:

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- the compatibility of technologies;

- the potential partner's acceptance of our approach to drug discovery;

- the novelty, quality and commercial potential of any drug candidate we may succeed in developing; and

- our ability, and collaborators' perceptions of our ability, to achieve intended results in a timely fashion, with acceptable quality and cost.

Even if we are able to gain the interest of potential drug development partners, 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 we develop on our own and, therefore, we may be unable to generate revenue from our products. In addition, our past, existing and future collaboration terms contain or will likely contain limitations on classes of chemical compounds or biological targets that we may explore outside those collaborations for our own use.

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 a 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.

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 in these industries);

- 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

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- 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 our drug 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 clinical trials and perform research and analysis services for us. 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 and clinical trial sites to ensure the proper and timely conduct of our clinical trials. While we have agreements governing their activities, we have limited influence over their actual performance. We have relied and plan to continue to rely upon CROs to monitor and manage our ongoing clinical programs, as well as the execution of nonclinical studies. We control only certain aspects of our CROs' activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with the FDA's current Good Clinical Practices, or cGCPs, which are regulations and guidelines enforced by the FDA for all of our product candidates in clinical development. The FDA enforces these cGCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our marketing applications. Upon inspection, the FDA may determine that our Phase 3 clinical trials do not comply with cGCPs. Our CROs are not our employees, and we cannot control whether or not they devote sufficient time and resources to our ongoing clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical studies, or other drug development activities which could harm our competitive position. 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 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. As a result, our financial results and the commercial prospects product candidates that we develop would be harmed, our costs could increase, and our ability to generate revenues could be delayed or eliminated.

We have limited manufacturing experience. Currently, we rely on third parties to provide sufficient quantities of our product candidates to conduct preclinical and clinical studies. We have no control over our manufacturers', suppliers' or collaborators' compliance with manufacturing regulations, and their failure to comply could interrupt our drug supply.

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 partners' 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 cGMP 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. If we or our partners are not able to obtain contract cGMP 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. There can be no assurance that we or our partners will be able to obtain such requisite terms, materials, technologies and

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intellectual property necessary to successfully manufacture our product candidates for clinical trials or commercialization. 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, 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 and our partners may undergo inspections by the FDA for compliance with cGMP regulations before our product candidates produced there can receive marketing approval. If these facilities do not satisfy cGMP requirements in connection with the manufacture of our product candidates, we and our partners 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. In addition, after approval of a product candidate for commercial use, our partners and contract manufacturers and any alternative contract manufacturer we and our partners may utilize will be subject to ongoing periodic inspection by the FDA and corresponding state and foreign agencies for compliance with cGMP regulations, similar foreign regulations and other regulatory standards. We and our partners do not have control over our contract manufacturers' compliance with these regulations and standards. Any failure by such third-party manufacturers or suppliers to comply with applicable regulations could result in sanctions being imposed (including fines, injunctions and civil penalties), 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.

Materials necessary to manufacture our product candidates currently under development may not be available on commercially reasonable terms, or at all, which may delay our development and commercialization of these drugs.

Some of the materials necessary for the manufacture of our product candidates currently under development may, from time to time, be available either in limited quantities, or from a limited number of manufacturers, or both. We and/or our collaborators need to obtain these materials for our clinical trials and, potentially, for commercial distribution when and if we obtain marketing approval for these compounds. Suppliers may not sell us these materials at the time we need them or on commercially reasonable terms. If we and/or our collaborators are unable to obtain the materials needed for the conduct of our clinical trials, product testing and potential regulatory approval could be delayed, adversely impacting our ability to develop the product candidates. If it becomes necessary to change suppliers for any of these materials or if any of our suppliers experience a shutdown or disruption in the facilities used to produce these materials, due to technical, regulatory or other problems, it could significantly hinder or prevent manufacture of our drug candidates and any resulting products.

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, biotechnology companies such as AbbVie Inc., Amgen, Inc., Ariad Pharmaceuticals, Inc., Astellas Pharma, Inc., Array BioPharma Inc., AstraZeneca PLC, Celgene Corporation, Curis, Inc., 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 087, we are aware of a number of companies that are or may be pursuing a number of different approaches to FGFR inhibition, including Ariad Pharmaceuticals, 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

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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, Novartis, Oncotherapy Services, Inc. and Spectrum Pharmaceuticals, Inc.

Regarding ARQ 092, 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 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., LOXO Oncology, Roche and Sunesis Pharmaceuticals. Moreover, numerous companies are also pursuing inhibitors of wild-type BTK, including AbbVie with its drug, IMBRUVICA™, and Astra Zeneca 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™.

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 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.

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RISKS RELATED TO INTELLECTUAL PROPERTY

Our patents and other proprietary rights may fail to protect our business. If we are unable to adequately protect our intellectual property, third parties may be able to use our technology which could adversely affect our ability to compete in the market.

To be successful and compete, we must obtain and protect patents on our products and technology and protect our trade secrets. 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. No consistent policy has emerged from the U.S. Patent and Trademark Office or the courts regarding the breadth of claims allowed or the degree of protection afforded under many biotechnology patents. In addition, there is a substantial backlog of biotechnology patent applications at the U.S. Patent and Trademark Office. As a consequence of these factors, the approval or rejection of patent applications may take several years.

Also, the Leahy-Smith America Invents Act was signed into law on September 16, 2011, and became fully effective in March 2013. In general, the legislation attempts to address issues surrounding the enforceability of patents and the increase in patent litigation by, among other things, moving to a first inventor-to-file system, establishing new procedures for challenging patents and establishing different methods for invalidating patents. 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 and prospects.

We do not know whether our patent applications will result in issued patents. 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.

Competitors may interfere with our patent protection in a variety of ways. Competitors may claim that they invented the claimed invention before us. Competitors may also claim that we are infringing on their patents and that, therefore, we cannot practice our technology as claimed under our patents. Competitors may also contest our patents by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our issued patents are not valid for a number of reasons. If a court agrees, our patents could be narrowed, invalidated or rendered unenforceable, or we may be forced to stop using the technology covered by these patents or to license the technology from third parties. As a company, we have no meaningful experience with competitors interfering with our patents or patent applications and therefore may not have the experience we would need to aggressively protect our patents should such action become necessary.

The laws of some foreign countries do not protect intellectual property rights to the same extent as the laws of the U.S. Many companies have encountered significant problems in protecting and defending such rights in foreign jurisdictions. Many countries, including certain countries in Europe, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of the patent. Compulsory licensing of life-saving drugs is also becoming increasingly popular in developing countries either through direct legislation or international initiatives. Such compulsory licenses could be extended to include some of our product candidates, which could limit our potential revenue opportunities. Moreover, the legal systems of certain countries, particularly certain developing countries, do not favor the aggressive enforcement of patent and other intellectual property protection, which makes it difficult to stop infringement.

Drug candidates we develop that are approved for commercial marketing by the FDA would 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

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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. It also provides a means for approving generic versions of a drug once the marketing exclusivity period has ended and all relevant patents have expired. The period of exclusive marketing, however, may be shortened if a patent is successfully challenged and defeated, which could reduce the amount of revenue we receive for such product.

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 copyright and trademark protection, trade secrets, and know-how. It is unclear whether our trade secrets and know-how will prove to be adequately protected. 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 U.S. are sometimes less willing than our federal and state courts to protect trade secrets. Furthermore, others may independently develop substantially equivalent knowledge, methods 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.

Our success will depend partly on our ability to operate without infringing upon or misappropriating the proprietary rights of others.

There are many patents in our field of technology and we cannot guarantee that we do not infringe on those patents or that we will not infringe on patents granted in the future. If a patent holder believes a product of ours infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology.

If we do not prevail in litigation or if other parties have filed, or in the future should file, patent applications covering products and technologies that we have developed or intend to develop, we may have to obtain licenses from third parties, which may not be available on commercially reasonable terms, or at all, and may require us to pay substantial royalties or grant a cross-license to some of our patents to another patent holder. Additionally, we may have to change the formulation of a product candidate so that we do not infringe third-party patents. Such reformulation may be impossible to achieve or which may require substantial time and expense. If we are unable to cost-effectively redesign our products so they do not infringe a patent, we may be unable to sell some of our products. Any of these occurrences will result in lost revenues and profits for us.

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. Legal proceedings relating to intellectual property would be expensive, take significant time and divert management’s attention from other business concerns. We face potential patent infringement suits by companies that control patents for drugs or potential drugs similar to our product candidates or other suits alleging infringement of their intellectual property rights. There could be issued patents of which we are not aware that our products and their use, whether as single agents or in combination with other products, infringe or patents that we believe we do not infringe that we are ultimately found to infringe. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patent applications can take many years to issue, there

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may be currently pending applications of which we are unaware that may later result in issued patents that we infringe with our drug candidates or resulting products, and their use as single agents or in combination with other 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 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.

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. 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 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 our facilities could be time consuming and result in substantial delays in our development of products and in fulfilling our agreements with our collaborators.

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 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. 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 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 PRODUCT LIABILITY

If our use of chemical and biological materials and hazardous materials violates applicable laws or causes personal injury, we may be liable for damages.

Our drug discovery activities, including the analysis and synthesis of chemical compounds, involve the controlled use of chemicals, including flammable, combustible, toxic and radioactive materials that are potentially hazardous if misused. Federal, state and local laws and regulations govern our use, storage, handling and disposal of these materials. These laws and regulations include the Resource Conservation and Recovery Act, the Occupational Safety and Health Act, local fire and building codes, regulations promulgated by the Department of Transportation, the Drug Enforcement Agency and the Department of Energy, the Department of Health and Human Services, and the laws of Massachusetts where we conduct our operations. We may incur significant costs to comply with these laws and regulations in the future and current or future environmental laws and regulations may impair our research, development and production efforts. Notwithstanding our extensive safety procedures for handling and disposing of materials, the risk of

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accidental contamination or injury from these materials cannot be completely eliminated. In the event of an accident, our business could be disrupted and we could be liable for damages. Our liability may exceed our insurance coverage and our total assets and have a negative impact on our financial condition and results of operations.

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.

RISKS RELATED TO OUR COMMON STOCK

Our stock price may be extremely volatile.

The trading price of our common stock has been highly volatile. As a result of this volatility, you may not be able to sell your common stock at or above your purchase price, if at all. We believe the trading price of our common stock will remain highly volatile and may fluctuate substantially due to factors such as:

- adverse results or delays in 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.

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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.

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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 rental rate 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.

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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, 2012 to December 31, 2017, 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, 2012. 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

	12/31/12	12/31/13	12/31/14	12/31/15	12/31/16	12/31/17
ArQule, Inc.	100.00	77.06	43.73	77.78	45.16	59.14
NASDAQ Market (U.S. Companies) Index	100.00	133.48	150.12	150.84	170.46	206.91
NASDAQ Biotechnology Index	100.00	165.97	223.07	249.32	196.09	238.51

ArQule's common stock is traded on the NASDAQ Global Market under the symbol "ARQL".

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	10,622,455	$ 3.01	5.74	450,494
Equity compensation plans not approved by stockholders	—	—	—	—

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The following table sets forth, for the periods indicated, the range of the high and low sale prices for ArQule's common stock:

	HIGH	LOW
2016		
First Quarter	$ 2.19	$ 1.37
Second Quarter	2.17	1.37
Third Quarter	1.94	1.39
Fourth Quarter	1.82	1.20
2017		
First Quarter	$ 1.68	$ 0.98
Second Quarter	1.44	0.92
Third Quarter	1.39	0.94
Fourth Quarter	1.70	0.97
2018		
First Quarter (through February 20, 2018)	$ 1.98	$ 1.46

As of February 20, 2018, there were approximately 77 holders of record and approximately 5,475 beneficial stockholders of our common stock.

Dividend Policy

We have never paid cash dividends on our common stock and we do not anticipate paying any cash dividends in the foreseeable future. We currently intend to retain future earnings, if any, for use in our business.

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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,				
	2017	2016	2015	2014	2013
STATEMENT OF OPERATIONS AND COMPREHENSIVE LOSS DATA:					
Revenue:					
Research and development revenue(a)	$ —	$ 4,709	$ 11,239	$ 11,254	$ 15,914
Costs and expenses:					
Research and development(b)	19,468	20,042	15,561	22,271	27,555
General and administrative	7,551	7,563	9,830	12,154	12,836
Restructuring and other costs(c)(d)	—	—	—	1,099	650
Total costs and expenses	27,019	27,605	25,391	35,524	41,041
Loss from operations	(27,019)	(22,896)	(14,152)	(24,270)	(25,127)
Interest income	238	178	101	272	502
Interest expense	(1,520)	—	—	(35)	(32)
Other income (expense)(e)	(902)	—	277	642	57
Loss before income taxes	(29,203)	(22,718)	(13,774)	(23,391)	$ (24,600)
Provision for income taxes	—	—	—	—	—
Net loss	(29,203)	(22,718)	(13,774)	(23,391)	(24,600)
Unrealized gain (loss) on marketable securities	(18)	(1)	13	(77)	(35)
Comprehensive loss	$ (29,221)	$ (22,719)	$ (13,761)	$ (23,468)	$ (24,635)
Basic and diluted net loss per share	$ (0.39)	$ (0.33)	$ (0.22)	$ (0.37)	$ (0.39)
Weighted average common shares outstanding—basic and diluted	74,813	69,714	62,808	62,627	62,480
Cash, cash equivalents and marketable securities(f)(g)	$ 48,036	$ 31,126	$ 38,772	$ 59,208	$ 74,695
Marketable securities-long term	—	—	—	2,058	20,391
	$ 48,036	$ 31,126	$ 38,772	$ 61,266	$ 95,086
Working capital(f)(g)	$ 38,824	$ 23,248	$ 28,661	$ 42,824	$ 53,883
Notes payable	14,607	—	—	—	1,700
Total assets	48,902	32,380	40,004	63,394	98,179
Total stockholders' equity(f)(g)	14,181	23,680	29,179	40,545	60,626

(a)

Revenue decreased in 2017 due to revenue decreases of $2.8 million from our Daiichi Sankyo tivantinib development agreement and $1.9 million from our Kyowa Hakko Kirin exclusive license agreement upon the completion of the

development period for both programs on December 31, 2016.

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

(b)

The $4.5 million increase in research and development expense in 2016 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.

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The $6.7 million decrease in research and development expense in 2015 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.

The $5.3 million decrease in research and development expense in 2014 was primarily due to lower labor related costs of $4.2 million from our July 2013 and August 2014 restructurings and reduced lab expenses of $1.0 million. Other cost reductions in 2014 of $0.9 million were partially offset by a $0.8 million increase in outsourced clinical and product development costs.

The $6.4 million decrease in research and development expense in 2013 was primarily due to lower labor related costs of $1.7 million from attrition and $1.0 million from the July 2013 restructuring, $1.1 million lower outsourced clinical and product development costs principally related to our Phase 1 and 2 programs for tivantinib, reduced lab expenses of $0.8 million, lower professional fees of $0.5 million, and other cost reductions of $1.3 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, such as the completion of the METIV-HCC trial. 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)

In July 2013, we implemented a focused reduction in our workforce of 26 positions, resulting in a remaining workforce of approximately 68 employees. This action was intended to align human and financial resources with our primary focus on clinical-stage development, while retaining our core discovery capabilities. The costs associated with this action were comprised of severance payments of $422 thousand and benefits continuation costs of $89 thousand all of which were paid by December 31, 2013. We also incurred non-cash charges of $139 thousand related to the modification of employee stock options.

(e)

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. Other income (expense) in 2013 includes a gain from the increase in fair value of our auction rate securities.

(f)

In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering the Company 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 2,260 shares of Series A Preferred (Warrants). Each share of Series A Preferred together with the associated Warrant is priced at $1,135 and will automatically convert into 1,000 shares of common stock upon the adoption of an amendment to the Company’s restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The Warrants have 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), are exercisable immediately and expire approximately four years from the date of the adoption of the amendment to the Company’s restated certificate of incorporation.

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.

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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 four years from the date of issuance

(g)

In February 2016, the Company 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 expired in March 2017.

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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 five 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 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 drugs, we intend 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 drugs if approved. The pipeline includes the following wholly-owned compounds:

- ARQ 531, an orally bioavailable, potent and reversible inhibitor of both wild type and C481S-mutant BTK, in Phase 1 for B-cell malignancies refractory to other therapeutic options;

- Miransertib (ARQ 092), a selective inhibitor of AKT, a serine/threonine kinase, in Phase 1/2 in rare Overgrowth Diseases and in Phase 1 for multiple oncology indications and in the rare disease, Proteus syndrome, in partnership with the National Institutes of Health (NIH);

- Derazantinib (ARQ 087), a multi-kinase inhibitor designed to preferentially inhibit the FGFR family of kinases, in a registrational trial in intrahepatic cholangiocarcinoma (iCCA) in patients with FGFR2 fusions;

- ARQ 751, a next-generation inhibitor of AKT, in Phase 1 for solid tumors harboring the AKT1 or PI3K mutation; and

- ARQ 761, a ß-lapachone analog being evaluated as a promoter of NQO1-mediated programmed cancer cell death, in Phase 1/2 in multiple oncology indications in partnership with The University of Texas Southwest Medical Center.

Tivantinib (ARQ 197), an orally administered, small molecule inhibitor of the c-Met receptor tyrosine kinase and its biological pathway is no longer being developed. We licensed commercial rights to tivantinib for human cancer

indications to Daiichi Sankyo Co., Ltd. (“Daiichi Sankyo”) in the U.S., Europe, South America and the rest of the world, excluding Japan and certain other Asian countries, where we licensed commercial rights to Kyowa Hakko Kirin Co., Ltd. (“Kyowa Hakko Kirin”).

We have incurred a cumulative deficit of approximately $534 million from inception through December 31, 2017. We recorded a net loss for 2015, 2016 and 2017 and expect a net loss for 2018.

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LIQUIDITY AND CAPITAL RESOURCES

	December 31,			% increase (decrease)	
	2017	2016	2015	2016 to 2017	2015 to 2016
	(in millions)				
Cash, cash equivalents and marketable securities short-term	$ 48.0	$ 31.1	$ 38.8	54%	(20)%
Working capital	38.8	23.2	28.7	67%	(19)%

	Year Ended December 31,		
	2017	2016	2015
	(in millions)		
Cash flow from:			
Operating activities	$ (25.2)	$ (22.9)	$ (22.2)
Investing activities	(11.9)	8.9	23.5
Financing activities	42.1	15.4	0.1

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 future services. For each of the years ended December 31, 2017, 2016, and 2015 our net use of cash was primarily driven by payments for operating expenses which resulted in net cash outflows of $25.2 million, $22.9 million, and $22.2 million, respectively.

Cash flow from investing activities. Our net cash used by investing activities of $11.9 million in 2017 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. Our net cash provided by investing activities of $23.5 million in 2015 was comprised of net maturities of marketable securities of $23.5 million, and proceeds from the sale of property and equipment of $0.3 million partially offset by purchases of property and equipment of $0.3 million. The composition and mix of cash, cash equivalents and marketable securities may change frequently as a result of the Company's 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 $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 net cash provided by financing activities of $0.1 million in the year ended December 31, 2015 consisted of cash inflows from employee stock plan purchases.

Our cash requirements may vary materially from those now planned depending upon 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

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expenditures, competitive and technological advances, acquisitions and other factors. We cannot guarantee that we will be able to develop any of our drug candidates into a commercial product. In January 2017, we entered into Loan Agreement with a principal balance of $15 million (see Note 8). The terms of the Loan Agreement required payments of interest on a monthly basis through September 2018 and payments of interest and principal from October 2018 to August 2021. 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 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 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 four years from the date of issuance. In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering the Company 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 2,260 shares of Series A Preferred (Warrants). Each share of Series A Preferred together with the associated Warrant is priced at $1,135 and will automatically convert into 1,000 shares of common stock upon the adoption of an amendment to the Company's restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The Warrants have 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), are exercisable immediately and expire approximately four years from the date of the adoption of the amendment to the Company's restated certificate of incorporation.

In February 2018 Roivant Sciences and ArQule, Inc. announced the initiation of a collaboration to pursue the development of derazantinib, a pan-FGFR (fibroblast growth factor receptor) inhibitor, in Greater China. As part of the collaboration, ArQule has granted a Roivant subsidiary (Sinovant) an exclusive license to develop and commercialize derazantinib in the People's Republic of China, Hong Kong, Macau, and Taiwan. Deal terms include an upfront payment to ArQule of $3 million and an additional $2.5 million development milestone within the first year. ArQule is also eligible for an additional $82 million in regulatory and sales milestones. Upon commercialization, ArQule will receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in the Greater China territory.

We anticipate that our cash, cash equivalents and marketable securities on hand at December 31, 2017, 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 the fourth quarter of 2019 which is in excess of 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.

Our contractual obligations were comprised of the following as of December 31, 2017 (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	$ —	$ 6,667	$ 9,233	$ —
Interest on notes payable	3,721	1,159	1,999	563	—
Operating lease obligations	1,333	560	773	—	—
Purchase obligations	5,409	5,409	—	—	—
Total	$ 26,363	$ 7,128	$ 9,439	$ 9,796	$ —

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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 thousand. The obligations for this facility are included in the table above.

The original maturity date of the notes payable was August 1, 2021 and in a February 2018 amendment was extended by one year to August 1, 2022 with principal payments 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 the Company's ongoing research and development efforts.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

A "critical accounting policy" is one which is both important to the portrayal of the Company's 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.

The Company policy is to utilize the milestone method to recognize substantive milestones.

Research and development payments associated with our collaboration agreements in effect prior to January 1, 2011 are recognized as research and development revenue using the contingency adjusted performance model. Under this model, when payments are earned, revenue is immediately recognized on a pro-rata basis in the period we achieve the milestone based on the time elapsed from inception of the agreement to the time the milestone is earned over the estimated duration of the development period under the agreement. Thereafter, the remaining portion of the milestone payment is recognized on a straight-line basis over the remaining estimated development period under the agreement. This estimated development period may ultimately be shorter or longer depending upon the outcome of the development work, resulting in accelerated or deferred recognition of the development revenue. Royalty payments will be recognized as revenue when earned. The costs associated with satisfying research and development contracts are included in research and development expense as incurred.

For our tivantinib collaboration with Daiichi Sankyo, we compared the collaboration costs we incurred with those of Daiichi Sankyo each quarter. If our costs for the quarter exceeded Daiichi Sankyo's we recognized revenue on the amounts due to us under the contingency adjusted performance model. Revenue was calculated on a pro-rata basis using the time elapsed from inception of the agreement over the estimated duration of the development period under the agreement. If our costs for the quarter were less than those of Daiichi Sankyo's, we reported the amount due to Daiichi Sankyo as contra-revenue in that quarter. Amounts recognized as contra-revenue are netted against our tivantinib Daiichi Sankyo research and development revenue. To the extent that our share of Phase 3 collaboration costs exceeded the amount of milestones and royalties received, that excess was netted against future milestones and royalties earned and was not reported as contra-revenue.

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

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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.

RESULTS OF OPERATIONS

The following are the results of operations for the years ended December 31, 2017, 2016 and 2015:

Revenue

				% increase (decrease)	
	2017	2016	2015	2016 to 2017	2015 to 2016
	(in millions)				
Research and development revenue	$ —	$ 4.7	$ 11.2	(58)%	—%

2017 as compared to 2016: Research and development revenue was zero in 2017 and in 2016 includes revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement. Revenue decreased in 2017 due to revenue decreases of $2.8 million from our Daiichi Sankyo tivantinib development agreement and $1.9 million from our Kyowa Hakko Kirin exclusive license agreement upon the completion of the development period for both programs on December 31, 2016.

2016 as compared to 2015: Research and development revenue in 2016 and 2015 includes revenue from the Daiichi Sankyo tivantinib development agreement and the Kyowa Hakko Kirin exclusive license agreement. Revenue decreased in 2016 due to revenue decreases of $2.7 million from our Daiichi Sankyo tivantinib development agreement and $3.8 million from our Kyowa Hakko Kirin exclusive license agreement. These decreases were due to the completion of the development period for both programs on December 31, 2016.

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Under the terms of our tivantinib collaboration agreement with Daiichi Sankyo we shared development costs equally with our share of Phase 3 costs funded solely from milestones and royalties, if any. In each quarter the tivantinib collaboration costs that we incurred were compared with those of Daiichi Sankyo. If our costs for the quarter exceeded Daiichi Sankyo's, we recognized revenue on the amounts due to us under the contingency adjusted performance model. Revenue was calculated on a pro-rata basis using the time elapsed from inception of the agreement over the estimated duration of the development period under the agreement. If our costs for the quarter were less than those of Daiichi Sankyo's, we reported the amount due to Daiichi Sankyo as contra-revenue in that quarter. To the extent that our share of Phase 3 collaboration costs exceeded the amount of milestones and royalties received, that excess was netted against milestones and royalties earned and was not reported as contra-revenue.

In the fourth quarter of 2013, following a recommendation by the Data Monitoring Committee that the METIV-HCC trial continue with patients receiving a lower dose of tivantinib than the dose originally employed in the trial, we reviewed the estimated development period and extended it to June 2016. On March 22, 2016, we and Daiichi Sankyo announced that the DMC of the METIV-HCC study conducted the planned interim assessment, and it was determined the trial would continue to its final analysis. Accordingly, we reviewed the estimated development period and extended it to December 2016. On February 17, 2017, we and Daiichi Sankyo announced that the METIV-HCC trial did not meet its primary endpoint of improving OS.

Our cumulative share of the Daiichi Sankyo Phase 3 costs through December 31, 2017 totaled $110.7 million. Our cumulative share of Phase 3 collaboration costs has exceeded the amount of milestones received through December 31, 2017 by $70.7 million which are not required to be repaid upon expiration of the agreement.

Research and development

				% increase (decrease)	
	2017	2016	2015	2016 to 2017	2015 to 2016
	(in millions)				
Research and development	$ 19.5	$ 20.1	$ 15.6	(3)%	29%

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.

2016 as compared to 2015: The $4.5 million increase in research and development expense in 2016 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. At December 31, 2016 and 2015, we had 21 employees dedicated to our research and development program.

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 that our research and development expense will remain significant 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.

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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 trials, we may elect to discontinue 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 generally varies substantially according to the type, complexity, novelty, and intended use of a product. It is not unusual for the preclinical and clinical development of each of these types of products to take nine years or more, and for total development costs to exceed $500 million for each 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 Daiichi Sankyo and Kyowa Hakko Kirin. 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 oncology 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 oncology 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.

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General and administrative

				% increase (decrease)	
	2017	2016	2015	2016 to 2017	2015 to 2016
	(in millions)				
General and administrative	$ 7.6	$ 7.6	$ 9.8	—%	(23)%

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

2016 compared to 2015: General and administrative expense in 2016 decreased by $2.2 million primarily due to lower facility costs of $1.6 million and labor related costs of $0.6 million. General and administrative headcount was 14 at December 31, 2016 and 15 at December 31, 2015.

Interest income, interest expense and other income (expense)

				% increase (decrease)	
	2017	2016	2015	2016 to 2017	2015 to 2016
	(in thousands)				
Interest income	$ 238	$ 178	$ 101	34%	76%
Interest expense	(1,520)	—	—	100%	—
Other income (expense)	(902)	—	277	100%	—

Interest income is comprised of interest income derived from our portfolio of cash, cash equivalents and investments. 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 income increased in 2016 primarily due to an increase in our portfolio balance resulting from our $15.2 million stock offering in February 2016.

Interest expense is from the loan agreement we entered into in January 2017.

Other income (expense) in 2017 includes a non-cash expense from an increase in fair value of our preferred stock warrant liability of $902 thousand. Other income (expense) in 2015 includes a gain of $277 thousand from the sale of property and equipment.

Provision for income taxes

There was no current or deferred tax expense for the years ended December 31, 2017, 2016 or 2015 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.

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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.

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ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

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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. as of December 31, 2017 and 2016 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, 2017, 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, 2017, 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, 2017 and 2016, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2017 in conformity with 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, 2017, based on criteria established in Internal Control—Integrated Framework (2013) issued by the COSO.

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. 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 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

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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 5, 2018

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

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ARQULE, INC.
BALANCE SHEETS

	December 31,	
	2017	2016
	(IN THOUSANDS, EXCEPT SHARE AND PER SHARE DATA)	
ASSETS		
Current assets:		
Cash and cash equivalents	$ 20,229	$ 15,267
Marketable securities-short term	27,807	15,859
Prepaid expenses and other current assets	547	822
Total current assets	48,583	31,948
Property and equipment, net	115	180
Other assets	204	252
Total assets	$ 48,902	$ 32,380
LIABILITIES, PREFERRED STOCK AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued expenses	$ 8,259	$ 8,700
Deferred revenue	1,500	—
Total current liabilities	9,759	8,700
Long-term liabilities:		
Notes payable	14,607	—
Warrant liability	1,512	—
Total liabilities	25,878	8,700
Commitments and contingencies (Note 13)		
Preferred stock, convertible, Series A $0.01 par value; 1,000,000, shares authorized; 8,370 and no shares issued and outstanding at December 31, 2017 and 2016, respectively, aggregate liquidation preference of $9,500	8,843	—
Stockholders' equity:		
Common stock, $0.01 par value; 100,000,000 shares authorized; 87,110,202 and 71,146,209 shares issued and outstanding at December 31, 2017 and 2016, respectively	871	711
Additional paid-in capital	547,364	527,802
Accumulated other comprehensive income (loss)	(16)	2
Accumulated deficit	(534,038)	(504,835)
Total stockholders' equity	14,181	23,680
Total liabilities, preferred stock and stockholders' equity	$ 48,902	$ 32,380

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

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ARQULE, INC.
STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

	YEAR ENDED DECEMBER 31,		
	2017	2016	2015
	(IN THOUSANDS, EXCEPT PER SHARE DATA)		
Revenue:			
Research and development revenue	$ —	$ 4,709	$ 11,239
Costs and expenses:			
Research and development	19,468	20,042	15,561
General and administrative	7,551	7,563	9,830
	27,019	27,605	25,391
Loss from operations	(27,019)	(22,896)	(14,152)
Interest income	238	178	101
Interest expense	(1,520)	—	—
Other income (expense)	(902)	—	277
Loss before income taxes	(29,203)	(22,718)	(13,774)
Provision for income taxes	—	—	—
Net loss	(29,203)	(22,718)	(13,774)
Unrealized gain (loss) on marketable securities	(18)	(1)	13
Comprehensive loss	$ (29,221)	$ (22,719)	$ (13,761)
Basic and diluted net loss per common share:			
Net loss per common share	$ (0.39)	$ (0.33)	$ (0.22)
Weighted average basic and diluted common shares outstanding	74,813	69,714	62,808

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

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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	STOCK-HOLDERS' EQUITY
	SHARES	AMOUNT	SHARES	PAR VALUE				
Balance at December 31, 2014	—	$ —	62,821,781	$ 628	$ 508,270	$ (10)	$ (468,343)	$ 40,545
Restricted shares issued net of forfeitures and shares redeemed for taxes			13,242	—				—
Employee stock purchase plan			104,757	1	133			134
Stock based compensation expense					2,261			2,261
Change in unrealized gain (loss) on marketable securities						13		13
Net loss							(13,774)	(13,774)
Balance at December 31, 2015	—	—	62,939,780	629	510,664	3	(482,117)	29,179
Issuance of common stock and options from stock offering, net			8,027,900	80	15,094			15,174
Stock option exercises and issuance of common stock			97,498	1	112			113
Restricted shares issued net of forfeitures and shares redeemed for			29,828	—				—

taxes								
Employee stock purchase plan			51,203	1	67			68
Stock based compensation expense					1,865			1,865
Change in unrealized gain (loss) on marketable securities						(1)		(1)
Net loss							(22,718)	(22,718)
Balance at December 31, 2016	—	—	71,146,209	711	527,802	2	(504,835)	23,680
Issuance of preferred stock and warrants from stock offering, net	8,370	8,843						
Issuance of common stock and warrants from stock offerings, net			15,938,651	160	17,764			17,924
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			1,434
Issuance of warrants from notes payable					347			347
Change in unrealized gain (loss) on marketable						(18)		(18)

securities								
Net loss							(29,203)	(29,203)
Balance at December 31, 2017	8,370	$ 8,843	87,110,202	$ 871	$ 547,364	$ (16)	$ (534,038)	$ 14,181

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

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ARQULE, INC.
STATEMENTS OF CASH FLOWS

	YEAR ENDED DECEMBER 31,		
	2017	2016	2015
	(IN THOUSANDS)		
Cash flows from operating activities:			
Net loss	$ (29,203)	$ (22,718)	$ (13,774)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	65	101	161
Amortization of premium (discount) on marketable securities	(28)	48	464
Amortization of deferred gain on sale leaseback	—	—	(232)
Amortization of debt discount	330	—	—
Change in fair value of warrant liability	902	—	—
Non-cash stock compensation	1,434	1,865	2,261
Gain on sale of property and equipment	—	—	(277)
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	322	(108)	1,029
Accounts payable and accrued expenses	(497)	2,466	(713)
Deferred revenue	1,500	(4,591)	(11,079)
Net cash used in operating activities	(25,175)	(22,937)	(22,160)
Cash flows from investing activities:			
Purchases of marketable securities	(41,971)	(30,975)	(36,978)
Proceeds from sale or maturity of marketable securities	30,033	39,856	60,479
Proceeds from sale of property and equipment	—	—	298
Purchases of property and equipment	—	(15)	(315)
Net cash provided by (used in) investing activities	(11,938)	8,866	23,484
Cash flows from financing activities:			
Proceeds from notes payable and warrants, net	14,624	—	—
Proceeds from common stock offerings and warrants, net	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	17	181	134
Net cash provided by financing activities	42,075	15,355	134
Net increase in cash and cash equivalents	4,962	1,284	1,458
Cash and cash equivalents, beginning of period	15,267	13,983	12,525
Cash and cash equivalents, end of period	$ 20,229	$ 15,267	$ 13,983

SUPPLEMENTAL DISCLOSURE OF CASH FLOW INFORMATION (IN THOUSANDS):
The Company paid interest on debt of $1,190 in 2017 and $0 in 2016 and 2015.
The Company paid no income taxes in 2017, 2016 or 2015.
The accompanying notes are an integral part of these financial statements.

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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 five 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 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 drugs, we intend 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 drugs if approved. The pipeline includes the following wholly-owned compounds:

- ARQ 531, an orally bioavailable, potent and reversible inhibitor of both wild type and C481S-mutant BTK, in Phase 1 for B-cell malignancies refractory to other therapeutic options;

- Miransertib (ARQ 092), a selective inhibitor of AKT, a serine/threonine kinase, in Phase 1/2 in rare Overgrowth Diseases and in Phase 1 for multiple oncology indications and in the rare disease, Proteus syndrome, in partnership with the National Institutes of Health (NIH);

- Derazantinib (ARQ 087), a multi-kinase inhibitor designed to preferentially inhibit the FGFR family of kinases, in a registrational trial in intrahepatic cholangiocarcinoma (iCCA) in patients with FGFR2 fusions;

- ARQ 751, a next-generation inhibitor of AKT, in Phase 1 for solid tumors harboring the AKT1 or PI3K mutation; and

- ARQ 761, a ß-lapachone analog being evaluated as a promoter of NQO1-mediated programmed cancer cell death, in Phase 1/2 in multiple oncology indications in partnership with The University of Texas Southwest Medical Center.

Tivantinib (ARQ 197), an orally administered, small molecule inhibitor of the c-Met receptor tyrosine kinase and its biological pathway is no longer being developed. We licensed commercial rights to tivantinib for human cancer indications to Daiichi Sankyo Co., Ltd. (“Daiichi Sankyo”) in the U.S., Europe, South America and the rest of the world, excluding Japan and certain other Asian countries, where we had licensed commercial rights to Kyowa Hakko Kirin

Co., Ltd. (“Kyowa Hakko Kirin”).
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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, 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.

Our cash requirements may vary materially from those now planned depending upon 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 drug candidates into a commercial product. In January 2017, we entered into a loan and security agreement (the “Loan Agreement”) with a principal balance of $15 million (see Note 8). The terms of the Loan Agreement require payments of interest on a monthly basis through September 2018 and payments of interest and principal from October 2018 to August 2021. The original maturity date of the loan was August 1, 2021 and in a February 2018 amendment was extended 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 $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 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 four years from the date of issuance. In November 2017, we entered into definitive securities purchase agreements with certain institutional investors. In conjunction with this stock offering the Company 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 2,260 shares of Series A Preferred (Warrants). Each share of Series A Preferred together with the associated Warrant is priced at $1,135 and will automatically convert into 1,000 shares of common stock upon the adoption of an amendment to the Company’s restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The Warrants have 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), are exercisable immediately and expire approximately four years from the date of the adoption of the amendment to the Company’s restated certificate of incorporation.

In February 2018 Roivant Sciences and ArQule, Inc. announced the initiation of a collaboration to pursue the development of derazantinib, a pan-FGFR (fibroblast growth factor receptor) inhibitor, in Greater China. As part of the collaboration, ArQule has granted a Roivant subsidiary (Sinovant) an exclusive license to develop and commercialize derazantinib in the People’s Republic of China, Hong Kong, Macau, and Taiwan. Deal terms include an upfront payment to ArQule of $3 million and an additional $2.5 million development milestone within the first year. ArQule is also eligible for an additional $82 million in regulatory and sales milestones. Upon commercialization, ArQule will receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in the Greater China territory.

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

1. ORGANIZATION AND NATURE OF OPERATIONS (Continued)

We anticipate that our cash, cash equivalents and marketable securities on hand at December 31, 2017, 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. The Company classifies its investments as either current or long-term based upon the investments' contractual maturities and the Company's 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, 2017 we had no trading securities.

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

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

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

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, 2017, 2016 or 2015. 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 assets of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

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

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

- Level 3—Unobservable inputs that are supported by little or no market activity 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, 2017 and 2016 our financial instruments consist of cash, cash equivalents, investments in corporate debt securities, accounts payable, notes payable and accrued expenses. At December 31, 2017 and 2016 our financial instruments also included marketable securities and warrant liabilities which are reported at fair value. The warrant liability is carried at fair value and determined to be a Level 3 liability in the fair value hierarchy described above. The carrying values of cash, cash equivalents, investments in corporate debt 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 the Company's notes payable approximates its 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, 2017, 2016 and 2015 was $65, $101 and $161, 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.

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

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

We recognize revenues when all of the following criteria are met: persuasive evidence of an arrangement exists; delivery has occurred or services have been rendered; our price to the customer is fixed or determinable; and collectability is reasonably assured.

We elected the milestone method of revenue recognition, which may impact any new collaboration agreements or material modifications to existing agreements, in the event we elect the policy of utilizing the milestone method to recognize future substantive milestones.

Research and development payments associated with the collaboration agreements in effect prior to January 1, 2011 were recognized as research and development revenue using the contingency adjusted performance model. Under this model, when payments are earned, revenue is immediately recognized on a pro-rata basis in the period we achieve the milestone based on the time elapsed from inception of the agreement to the time the milestone is earned over the estimated duration of the development period under the agreement. Thereafter, the remaining portion of the milestone payment is recognized on a straight-line basis over the remaining estimated development period under the agreement. This estimated development period may ultimately be shorter or longer depending upon the outcome of the development work, resulting in accelerated or deferred recognition of the development revenue. Royalty payments will be recognized as revenue when earned. The costs associated with satisfying research and development contracts are included in research and development expense as incurred.

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 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 and in 2015 includes a gain of $277 from the sale of property and equipment.

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

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

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, the Company recognizes 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.

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. Potential common shares, for the year ended December 31, 2015, include 8,305,950 shares that would be issued upon the exercise of outstanding employee and Board of Director stock options.

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.

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2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

The following table presents stock-based compensation expense for the years ended December 31, 2017, 2016 and 2015 included in our Statements of Operations and Comprehensive Loss:

	2017	2016	2015
Research and development	$ 359	$ 514	$ 682
General and administrative	1,075	1,351	1,579
Total stock-based compensation expense	$ 1,434	$ 1,865	$ 2,261

In the years ended December 31, 2017, 2016 and 2015, no stock-based compensation expense was capitalized and there were no recognized tax benefits associated with the stock-based compensation charges.
The fair value of stock options and employee stock purchase plan shares granted in the years ended December 31, 2017, 2016 and 2015 respectively were estimated on the grant date using the Black-Scholes option-pricing model with the following assumptions:

	2017	2016	2015
Dividend yield(1)	0.0%	0.0%	0.0%
Weighted average expected volatility factor(2)	62%	63%	66%
Risk free interest(3)	1.9–2.1%	1.2–1.8%	1.3–1.7%
Expected term, excluding options issued pursuant to the Employee Stock Purchase Plan(4)	6.1–7.1 years	5.8–7.2 years	5.6–6.8 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.

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2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

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 will be effective for us on January 1, 2018. The adoption of this standard is not expected to 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 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 will be effective for us on January 1, 2018. The adoption of this standard is not expected to 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). The new standard requires that all lessees recognize the assets and liabilities that arise from leases on the balance sheet and disclose qualitative and quantitative information about its leasing arrangements. The new standard will be effective for us on January 1, 2019. We are currently evaluating the potential impact that this standard may have on our financial position and results of operations.

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. The standard is effective prospectively for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2016, with early adoption permitted. This new standard became effective for us on January 1, 2017. We adopted this standard in 2017 and determined that this ASU 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. This new standard requires a company to recognize revenue 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. The FASB subsequently issued amendments to ASU No. 2014-09 that have the same effective date and transition date. These new standards became effective for us on January 1, 2018, and will be adopted using the modified retrospective method through a

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2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES (Continued)

cumulative-effect adjustment directly to retained earnings as of that date. We have performed a review of these new standards as compared to our current accounting policies for customer contracts and collaborative relationships. During the fourth quarter of 2017 we finalized our assessments over the impact that these new standards will have on our consolidated results of operations, financial position and disclosures. As of December 31, 2017, we have not identified any accounting changes that would materially impact the amount of reported revenues with respect to our revenues from collaboration agreements; however, the adoption of these new standards may result in a change in the timing of revenue recognition related to certain of our licensing activities. As of December 31, 2017, we expect to recognize an adjustment of $1.5 million to retained earnings reflecting the cumulative impact for the accounting changes related to certain license arrangements made upon adoption of these new standards.

3. COLLABORATIONS AND ALLIANCES

Daiichi Sankyo Tivantinib Agreement

As previously reported, on December 18, 2008, we entered into a license, co-development and co-commercialization agreement with Daiichi Sankyo to conduct research, clinical trials and the commercialization of tivantinib in human cancer indications in the U.S., Europe, South America and the rest of the world, excluding Japan, China (including Hong Kong), South Korea and Taiwan, where Kyowa Hakko Kirin has exclusive rights for development and commercialization.

Under the terms of our tivantinib collaboration agreement with Daiichi Sankyo we shared development costs equally with our share of Phase 3 costs funded solely from milestones and royalties. In each quarter the tivantinib collaboration costs we incurred were compared with those of Daiichi Sankyo. If our costs for the quarter exceeded Daiichi Sankyo's, we recognized revenue on the amounts due to us under the contingency adjusted performance model. Revenue was calculated on a pro-rata basis using the time elapsed from inception of the agreement over the estimated duration of the development period under the agreement. If our costs for the quarter were less than those of Daiichi Sankyo, we reported the amount due to Daiichi Sankyo as contra-revenue in that quarter. To the extent that our share of Phase 3 collaboration costs exceeded the amount of milestones and royalties received, that excess was netted against milestones and royalties earned and was not reported as contra-revenue.

Our cumulative share of the Daiichi Sankyo Phase 3 costs through December 31, 2017 totaled $110.7 million. Our cumulative share of Phase 3 collaboration costs has exceeded the amount of milestones received through December 31, 2017 by $70.7 million which are not required to be repaid upon expiration of the agreement.

On March 22, 2016, we and Daiichi Sankyo announced that the DMC of the METIV-HCC study conducted the planned interim assessment, and it was determined the trial would continue to its final analysis. Accordingly, we reviewed the estimated development period and extended it to December 2016. On February 17, 2017, we and Daiichi Sankyo announced that the METIV-HCC trial did not meet its primary endpoint of improving OS. On February 17, 2017, we and Daiichi Sankyo announced that the METIV-HCC trial did not meet its primary end point of improving OS. As a result, Daiichi Sankyo and we have discontinued development of tivantinib.

No revenue was recognized in 2017. For the years ended December 31, 2016 and 2015, $2.8 million, net of $0.1 million of contra-revenue and $5.5 million, net of $0.1 million of contra-revenue, respectively, was recognized as revenue. At December 31, 2017 and 2016, there was no deferred revenue related to this agreement.

Kyowa Hakko Kirin Licensing Agreement

As previously reported, on April 27, 2007, we entered into an exclusive license agreement with Kyowa Hakko Kirin to develop and commercialize tivantinib in Japan and parts of Asia. Revenue for this

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3. COLLABORATIONS AND ALLIANCES (Continued)

agreement was recognized using the contingency-adjusted performance model with an estimated development period through December 31, 2016. On March 27, 2017, we reported that Kyowa Hakko Kirin announced top-line results of the JET-HCC Phase 3 trial of tivantinib in Japan, and that the trial did not meet its primary endpoint of improving PFS. Our joint development of tivantinib has subsequently been discontinued. As a result, Kyowa Hakko Kirin has discontinued development of tivantinib in the Asian territory.

Revenue for this agreement was recognized using the contingency-adjusted performance model with an estimated development period that ended on December 31, 2016. For the years ended December 31, 2016 and 2015, $1.9 million and $5.7 million, respectively, was recognized as revenue. At December 31, 2017 and 2016 there was no deferred revenue related to this agreement.

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. We have recorded deferred revenue of $1.5 million related to this licensing agreement.

Roivant Sciences Licensing Agreement

In February 2018 Roivant Sciences and ArQule, Inc. announced the initiation of a collaboration to pursue the development of derazantinib, a pan-FGFR inhibitor, in Greater China. As part of the collaboration, ArQule has granted a Roivant subsidiary (Sinovant) an exclusive license to develop and commercialize derazantinib in the People's Republic of China, Hong Kong, Macau, and Taiwan. Deal terms include an upfront payment to ArQule of $3 million and an additional $2.5 million development milestone to be paid within the first year. ArQule is also eligible for an additional $82 million in regulatory and sales milestones. Upon commercialization, ArQule will receive double digit royalties in the low teens from Sinovant on net sales of derazantinib in the Greater China territory.

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

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4. MARKETABLE SECURITIES AND FAIR VALUE MEASUREMENTS (Continued)

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, 2017 and December 31, 2016:

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

December 31, 2016	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Security type				
Corporate debt securities-short term	$ 15,857	$ 7	$ (5)	$ 15,859
Total available-for-sale marketable securities	$ 15,857	$ 7	$ (5)	$ 15,859

None of our available-for-sale marketable securities were in a continuous unrealized loss position for more than 12 months at December 31, 2017 or 2016.

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	$ —

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4. MARKETABLE SECURITIES AND FAIR VALUE MEASUREMENTS (Continued)

	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. The assumptions used may change as the underlying sources of these assumptions and market conditions change. Based on the Black-Scholes model, the Company recorded a preferred stock warrants liability of $1,512 at December 31, 2017.

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 table presents information about our assets and liabilities that are measured at fair value on a recurring basis for the year ended December 31, 2016 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, 2016:

	December 31, 2016	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Cash equivalents	$ 12,923	$ 12,923	$ —	$ —
Corporate debt securities-short term	15,859	—	15,859	—
Total	$ 28,782	$ 12,923	$ 15,859	$ —

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5. PROPERTY AND EQUIPMENT

Property and equipment consist of the following at December 31, 2017 and 2016:

	USEFUL LIFE ESTIMATED (YEARS)	2017	2016
Machinery and equipment	5	$ 1,939	$ 1,939
Leasehold improvements	3–5	232	232
Furniture and fixtures	7	40	40
Computer equipment	3	2,497	2,497
		4,708	4,708
Less: Accumulated depreciation and amortization		4,593	4,528
Net property and equipment		$ 115	$ 180

In January 2015, we entered into a lease agreement for our headquarters facility in Burlington, MA 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 rental rate of $455.

6. OTHER ASSETS

Other assets include the following at December 31, 2017 and 2016:

	2017	2016
Security deposits	$ 204	$ 252
Total other assets	$ 204	$ 252

7. ACCOUNTS PAYABLE AND ACCRUED EXPENSES

Accounts payable and accrued expenses include the following at December 31, 2017 and 2016:

	2017	2016
Accounts payable	$ 537	$ 710
Accrued payroll	1,448	1,856
Accrued outsourced preclinical and clinical fees	5,409	5,461
Accrued professional fees	492	363
Other accrued expenses	373	310
	$ 8,259	$ 8,700

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, 2017 was 8.22%. The Loan Agreement required

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8. LOAN AGREEMENT (Continued)

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. We have considered the amended maturity as of December 31, 2017.

The expected remaining repayment of the $15 million loan principal at December 31, 2017 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 (i) 3% of the outstanding principal balance if prepayment occurs in months 1-12 following the closing, (ii) 2.0% of the outstanding principal balance in months 13-24 following the closing, and (iii) 1% thereafter.

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, 2017.

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 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.

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8. LOAN AGREEMENT (Continued)

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 the Company 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 2,260 shares of Series A Preferred (Warrants). Each share of Series A Preferred together with the associated Warrant is priced at $1,135 and will automatically convert into 1,000 shares of common stock upon the adoption of an amendment to the Company's restated certificate of incorporation to increase the number of authorized shares of common stock thereunder. The amount reported as preferred stock at December 31, 2017 is $8.9 million which is net of the $0.6 million warrant liability established on the date of issuance in November 2017.

The Warrants have 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), are exercisable immediately and expire approximately four years from the date of the adoption of the amendment to the Company's restated certificate of incorporation. The fair value of the warrants on the date they were issued was $0.6 million which was recorded as a warrant liability. 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. Upon conversion of the preferred stock to common stock the warrant liability will be extinguished with an offsetting amount included as additional paid-in capital in stockholders' equity.

In November 2017, the Company issued Series A preferred stock and warrants to purchase shares of Series A preferred stock for aggregate net proceeds of $9.5 million. The terms of the Series A preferred, specifically the terms of the liquidation preference, require 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 preferred stock 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 is marked to market value through the income statement as a non-cash gain or loss at each reporting period.

The Series A Preferred Stockholders vote on an as converted basis together as one class with the holders of common stock.

If declared by the board, the Series A Preferred are eligible for a dividend on an as-converted basis. The Series A Preferred automatically converts into common stock upon the adoption of an amendment to the Company's restated certificate of incorporation to increase the number of authorized shares of common stock. If the Company's restated certificate of incorporation has not been adopted by July 1, 2018, the Series A Preferred will obtain a dividend in kind until such time as the restated certificate of incorporation is adopted. In the case of a liquidation event or deemed liquidation event defined by the definitive securities purchase agreements the holders of Series A Preferred Stock have a liquidation preference on the greater of the Series A Preferred Stock stated value or the consideration that would have been paid on such Series A Preferred Stock in the applicable liquidation event.

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10. COMMON STOCK

Our amended Certificate of Incorporation authorizes the issuance of up to 100 million shares of $0.01 par value common stock.

At December 31, 2017, we have 450,494 shares reserved for future issuance of common stock options pursuant to the 2014 Equity Incentives Plan ("Equity Incentives Plan").

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 four years from the date of issuance.

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. 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, 2017, no stock appreciation rights have been issued. At December 31, 2017, there were 450,494 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 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 2017. Additionally, under the 2014 Equity Incentives Plan options issued to Directors to purchase 280,000 shares of common stock are outstanding at December 31, 2017 of which 125,000 are exercisable.

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

11. EQUITY INCENTIVE PLANS (Continued)

Option activity under the Plans for the year ended December 31, 2017 was as follows:

Stock Options	Number of Shares	Weighted Average Exercise Price
Outstanding as of December 31, 2016	8,715,048	$ 3.71
Granted	2,572,500	1.19
Exercised	—	—
Cancelled	(665,093)	5.16
Outstanding as of December 31, 2017	10,622,455	$ 3.01
Exercisable as of December 31, 2017	6,342,885	$ 4.10

The following table summarizes information about options outstanding at December 31, 2017:

	Options Outstanding			Options Exercisable	
Range of Exercise Prices	Number Outstanding at December 31, 2017	Weighted Average Remaining Contractual Life	Weighted Average Exercise Price	Exercisable as of December 31, 2017	Weighted Average Exercise Price
$1.16–1.41	2,572,702	8.36	$ 1.05	458,102	$ 1.16
1.42–2.35	2,756,150	8.21	1.69	776,600	1.73
2.36–3.80	2,407,565	4.05	2.88	2,222,145	2.90
3.81–5.60	982,650	0.64	4.18	982,650	4.18
5.61–8.40	1,903,388	3.41	7.13	1,903,388	7.13
	10,622,455	5.74	$ 3.01	6,342,885	$ 4.10

The aggregate intrinsic value of options outstanding at December 31, 2017 was $1,687. The weighted average grant date fair value of options granted in year ended December 31, 2017, 2016 and 2015 was $0.72, $1.10, and $0.77, per share, respectively. In the year ended December 31, 2017 no options were exercised. In the year ended December 31, 2016 97,498 options were exercised.
Options vested, expected to vest and exercisable at December 31, 2017 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, 2017	10,415,678	$ 3.01	5.74	$ 1,629
Exercisable at December 31, 2017	6,342,885	$ 4.10	3.88	$ 247

The total compensation cost not yet recognized as of December 31, 2017 related to non-vested option awards was $2,287 which will be recognized over a weighted-average period of 2.5 years. During the year ended December 31, 2017, 198,513 shares were forfeited with a weighted average grant date fair value of $0.97 per share and a weighted average exercise price of $1.63 per share. During the year ended December 31, 2017, 466,580 shares expired with a weighted average grant date fair value of $3.62 per share and a weighted average exercise price of $6.66 per share. The weighted average remaining contractual life for options exercisable at December 31, 2017 was 3.9 years.

In 2013, we granted 242,697 shares of restricted stock to employees, vesting annually over a four-year period. No restricted stock was granted in 2017, 2016, 2015 or 2014. The weighted average fair value of the restricted stock at the time of grant in 2013 was $2.51 per share, and is being expensed ratably over the

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ARQULE, INC.
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11. EQUITY INCENTIVE PLANS (Continued)

vesting period. We recognized share-based compensation expense related to restricted stock of $8, $73 and $76 for the year ended December 31, 2017, 2016 and 2015, respectively.

Restricted stock activity under the equity incentives plan for the year ended December 31, 2017 was as follows:

Restricted Stock	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2016	29,276	$ 2.51
Granted	—	—
Vested	(29,276)	2.51
Cancelled	—	2.51
Unvested as of December 31, 2017	—	$ 2.51

The fair value of restricted stock vested in 2017, 2016 and 2015 was $48, $55 and $78, respectively.

In April 2017, the Company amended its chief executive officer's (the "CEO's") and chief operating officer's (the "COO's") employment agreements to grant 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, the Company amended its chief medical officer's (the "CMO's") employment agreement to grant 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. Through December 31, 2017 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 the Company's 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 2017. We recognized share-based compensation expense related to the 1996 Employee Stock Purchase Plan of $17, $60 and $60 for the year ended December 31, 2017, 2016 and 2015, respectively.

12. INCOME TAXES

There was no current or deferred tax expense for the years ended December 31, 2017, 2016 or 2015 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.

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ARQULE, INC.
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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, 2017, 2016 and 2015:

	2017	2016	2015
Income tax (benefit) expense at statutory rate	$ (9,929)	$ (7,724)	$ (4,683)
State tax (benefit) expense, net of Federal tax (benefit) expense	(1,452)	(1,146)	(677)
Permanent items	508	263	239
Effect of change in valuation allowance and State NOL expiration	(39,972)	8,837	4,945
Tax credits	(544)	(228)	(74)
Change in tax rate on beginning assets	51,445	—	—
Other	(56)	(2)	250
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, 2017 and 2016:

	2017	2016
Deferred tax assets:		
Net operating loss carryforwards	$ 100,421	$ 135,642
Tax credit carryforwards	26,771	25,264
Equity based compensation	3,381	9,610
Book depreciation in excess of tax	49	56
Reserves and accruals	171	181
Other	16	28
	130,809	170,781
Valuation allowance	(130,809)	(170,781)
Deferred tax liabilities	—	—
Net deferred tax assets	$ —	$ —

Total valuation allowance decreased by $39,972 for the year ended December 31, 2017, and increased by $8,837 and $4,945 for the years ended December 31, 2016 and 2015, respectively. 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, 2017. The Tax Cuts and Jobs Act was enacted on December 22, 2017. The Act reduces the US federal corporate tax rate from 35% to 21%, requires companies to re-measure the deferred tax assets and deferred tax liabilities as of the date of enactment. 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 is offset by the full valuation allowance.

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ARQULE, INC.
NOTES TO FINANCIAL STATEMENTS (Continued)
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12. INCOME TAXES (Continued)

As of December 31, 2017, we had federal NOL, state NOL, and research and development credit carryforwards of approximately $409,409, $228,565 and $28,253 respectively, which expire at various dates through 2037. Approximately $15,080 of our federal NOL and $929 of our state NOL are attributable to stock-based compensation windfall deductions as of 12/31/2016. We recorded a deferred tax asset for previously unrecognized excess tax benefit, offset by valuation allowance upon the adoption in 2017 of ASU 2016-09, “Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting.”

As of December 31, 2017, and 2016 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, 2017 and 2016, we had no accrued interest or penalties related to uncertain tax positions. Our U.S. federal tax returns for the tax years 2013 through 2017 and our state tax returns for the tax years 2013 through 2017 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, 2018, 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
2018	$ 560
2019	519
2020	254
Thereafter	—
Total minimum lease payments	$ 1,333

Rent expense under our non-cancelable operating lease was approximately $546, $540, and $1,569 for the years ended December 31, 2017, 2016 and 2015, respectively.

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NOTES TO FINANCIAL STATEMENTS (Continued)
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13. COMMITMENTS AND CONTINGENCIES (Continued)

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.

14. CONCENTRATION OF CREDIT RISK

Revenue from one customer represented approximately 40% of total revenue during 2016 and 49% in 2015. Revenue from another customer represented approximately 60% of total revenue during 2016 and 51% in 2015. There were no revenues recognized in 2017.

15. SELECTED QUARTERLY FINANCIAL DATA (UNAUDITED)

	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)

	FIRST QUARTER	SECOND QUARTER	THIRD QUARTER	FOURTH QUARTER
2016				
Net revenues	$ 1,227	$ 1,072	$ 1,223	$ 1,187
Net loss	(4,981)	(5,100)	(5,817)	(6,820)
Basic and diluted earnings (loss) per share:				
Net loss per share	$ (0.08)	$ (0.07)	$ (0.08)	$ (0.10)

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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, 2017. 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, 2017, 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.

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, 2017.

The effectiveness of our internal control over financial reporting as of December 31, 2017 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 quarter ended December 31, 2017 that has materially affected or is reasonably likely to materially affect our internal control over financial reporting.

ITEM 9B.
OTHER INFORMATION

None.

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PART III

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 8, 2018, 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 the executive officers of the Company 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 the Company's 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.

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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 as Exhibit 3.1 to the Company's Annual Report on Form 10-K filed on March 2, 2011 (File No. 000-21429) and incorporated herein by reference.
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.
3.3	Certificate of Designations dated November 7, 2017 for the Convertible Series A Preferred Stock as filed with the Secretary of State of the State of Delaware Filed as Exhibit 3.1 to the Company's Current Report on Form 8-K filed on November 8, 2017 (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	Warrant dated January 6, 2017 issued to Oxford Finance LLC. Filed as Exhibit 4.1 to the Company's Current Report on Form 8-K filed on January 10, 2017 (File No. 000-21429) and incorporated herein by reference.
4.3	Warrant dated January 6, 2017 issued to Oxford Finance LLC. Filed as Exhibit 4.2 to the Company's Current Report on Form 8-K filed on January 10, 2017 (File No. 000-21429) and incorporated herein by reference.
4.4	Form of 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.
4.5	Form of Warrant. Filed as Exhibit 4.1 to the Company's Current Report on Form 8-K filed on November 8, 2017 (File No. 000-21429) and incorporated herein by reference.
4.6	Form of Warrant. 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*	Amended and Restated 1996 Employee Stock Purchase Plan. Filed as Appendix B to the Company's Definitive Proxy Statement filed on April 29, 2011 (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*	2005 Director Stock Compensation Plan. Filed as Exhibit 4 to the Company's Registration Statement on Form S-8 filed on December 6, 2005 (File No. 333-130159) and incorporated herein by reference.
10.5*	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.
10.6+	

Exclusive License Agreement by and between the Company and Kyowa Hakko Kogyo Co., Ltd. Filed as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2007 filed on August 7, 2007 (File No. 000-21429) and incorporated herein by reference.

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EXHIBIT NO.	DESCRIPTION
10.7*	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.
10.8*	Form of Incentive Stock Option Agreement. 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.
10.9*	Form of Non-Statutory Stock Option Agreement. 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.
10.10*	Second Amendment to Employment Agreement, dated April 14, 2008, by and between ArQule, Inc. and Peter S. Lawrence. 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.
10.11*	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.
10.12*	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.
10.13*	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.
10.14*	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.
10.15*	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.
10.16*	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.
10.17*	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.
10.18*	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.
10.19*	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.
10.20*	2014 Equity Incentives Plan. Filed as Appendix B to the Company's Definitive Proxy Statement filed on April 11, 2014 (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 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.
10.22*	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.

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EXHIBIT NO.	DESCRIPTION
10.23*	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.
10.24	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.
10.25*	Form of Performance-based Option Agreement. 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.
10.26*	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.
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	Form of Securities Purchase Agreement. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on October 16, 2017 (File No. 000-21429) and incorporated herein by reference.
10.30+	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.
10.31+	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.
10.32+	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.
10.33	Form of Securities Purchase Agreement. Filed as Exhibit 10.1 to the Company's Current Report on Form 8-K filed on November 8, 2017 (File No. 000-21429) and incorporated herein by reference.
10.34	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.
10.35+	License Agreement, dated February 2, 2018, by and among ArQule, Inc., Sinovant Sciences Ltd. and Roivant Sciences Ltd., filed herewith.
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.

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EXHIBIT NO.	DESCRIPTION
101	The following materials from ArQule, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2017, 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.

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SIGNATURES

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

ArQule, Inc.

By: /s/ Paolo Pucci

Paolo Pucci

Chief Executive Officer

Date: March 5, 2018

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 5, 2018
/s/ Peter S. Lawrence Peter S. Lawrence	President and Chief Operating Officer (Principal Financial Officer)	March 5, 2018
/s/ Robert J. Weiskopf Robert J. Weiskopf	Chief Financial Officer and Treasurer (Principal Accounting Officer)	March 5, 2018
/s/ Patrick J. Zenner Patrick J. Zenner	Director—Chairman of the Board	March 5, 2018
/s/ Timothy C. Barabe Timothy C. Barabe	Director	March 5, 2018
/s/ Susan L. Kelley Susan L. Kelley	Director	March 5, 2018
/s/ Ronald M. Lindsay Ronald M. Lindsay	Director	March 5, 2018
/s/ Michael D. Loberg Michael D. Loberg	Director	March 5, 2018
/s/ William G. Messenger William G. Messenger	Director	March 5, 2018
/s/ Ran Nussbaum Ran Nussbaum	Director	March 5, 2018