

Mirati Therapeutics, Inc.  
Form 10-K  
March 08, 2018  
UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549

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FORM 10-K

(Mark one)

☒ ANNUAL REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.

For the fiscal year ended December 31, 2017; or

☐ TRANSITION REPORT UNDER SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number: 1-15803

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MIRATI THERAPEUTICS, INC.

(Exact Name of Registrant as Specified in Its Charter)

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|                                                                   |                                      |
|-------------------------------------------------------------------|--------------------------------------|
| Delaware                                                          | 46-2693615                           |
| (State or other jurisdiction of<br>incorporation or organization) | (IRS Employer<br>Identification No.) |

|                                                          |            |
|----------------------------------------------------------|------------|
| 9393 Towne Centre Drive Suite 200, San Diego, California | 92121      |
| (Address of principal executive offices)                 | (Zip Code) |

Registrant's telephone number: (858) 332-3410

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

| Title of Each Class                       | Name of Each Exchange on Which Registered |
|-------------------------------------------|-------------------------------------------|
| Common Stock, Par value \$0.001 per share | The NASDAQ Stock Market LLC               |

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

None

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

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

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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 the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer ☐ Accelerated filer ☐

Non-accelerated filer ☒ (Do not check if a smaller reporting company) Smaller reporting company ☐

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financing 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 common stock held by non-affiliates (based on the closing price on the last business day of the registrant's most recently completed second fiscal quarter as reported on the NASDAQ Capital Market) was \$74 million. All executive officers and directors of the registrant and all persons filing a Schedule 13D or Schedule 13G with the Securities and Exchange Commission in respect to registrant's common stock have been deemed, solely for the purpose of the foregoing calculation, to be "affiliates" of the registrant.

As of March 5, 2018, the registrant had 28,940,161 shares of common stock outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Certain information required to be disclosed in Part III of this report is incorporated by reference from the registrant's definitive Proxy Statement for the 2018 Annual Meeting of Stockholders, which will be held on May 16, 2018 and which proxy statement will be filed not later than 120 days after the end of the fiscal year covered by this report.

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## PART I

### Forward-Looking Statements

This Annual Report on Form 10-K (the "Annual Report") may contain "forward-looking statements" within the meaning of the federal securities laws made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth under Part I, Item 1A, "Risk Factors" in this Annual Report. Except as required by law, we assume no obligation to update these forward-looking statements, whether as a result of new information, future events or otherwise. These statements, which represent our current expectations or beliefs concerning various future events, may contain words such as "may," "will," "expect," "anticipate," "intend," "plan," "believe," "estimate" or other words indicating future results, though not all forward-looking statements necessarily contain these identifying words. Such statements may include, but are not limited to, statements concerning the following:

- the initiation, cost, timing, progress and results of our research and development activities, preclinical studies and future clinical trials;
- our ability to obtain and maintain regulatory approval for our product candidates, and any related restrictions, limitations, and/or warnings in the label of any approved product candidate;
- our ability to obtain funding for our operations;
- our plans to research, develop and commercialize our product candidates;
- our strategic partners' decisions relating to development and commercialization of product candidates;
- our ability to attract collaborators with development, regulatory and commercialization expertise;
- our ability to obtain and maintain intellectual property protection for our product candidates;
- the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- our ability to successfully commercialize our product candidates;
- the rate and degree of market acceptance of our product candidates;
- our ability to develop sales and marketing capabilities, whether alone or with potential future collaborators;
- regulatory developments in the United States and foreign countries;
- the performance of our third-party suppliers and manufacturers;
- the success of competing therapies that are or become available;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012 ("the JOBS Act");
- the loss of key scientific or management personnel; and

· our future financial results, capital requirements and need for additional financing.

References in this Annual Report on Form 10-K to "we", "our", "us", "Mirati" or "the Company" refer to Mirati Therapeutics, Inc. and its subsidiaries.

Item 1. Business

**BUSINESS**

Overview

Mirati Therapeutics, Inc. is a clinical-stage oncology company developing targeted therapeutics to address the genetic, epigenetic and immunological promoters of cancer. Our precision oncology clinical programs utilize next-generation genomic testing to identify and select cancer patients who are most likely to benefit from targeted treatment. In immuno-oncology, we are advancing clinical programs where the ability of our product candidates to improve the immune environment of tumor cells may enhance and expand the efficacy of existing cancer immunotherapy medicines when given in combination. Our preclinical programs include product candidates specifically designed to address genetic mutations in cancer patients where few treatment options exist. We approach each of our discovery and development programs with a singular focus: to translate our deep understanding of the molecular drivers of cancer into better therapies and better outcomes for patients.

Our clinical and preclinical programs are summarized in the chart below:

Sitravatinib

Sitravatinib in Combination with Immune Checkpoint Inhibitors

Background

Sitravatinib is an oral, spectrum-selective kinase inhibitor that potently inhibits specific receptor tyrosine kinases (“RTKs”). RTKs are families of kinases involved in the transmission of signals that regulate cell growth, survival, and migration and include TAM family receptors (TYRO3, Axl, Mer), split family receptors (VEGFR2, KIT) and RET. Sitravatinib addresses cancer via two distinct mechanisms: directly, by targeting RTKs that drive tumor growth through mutation, and indirectly, by

modulating immune regulatory cells to stimulate the body's immune response to tumors. Sitravatinib's potent inhibition of TAM and split family receptors may help overcome resistance to immune checkpoint inhibitors and stimulate the body's immune response to help detect and destroy tumor cells. Blocking the signaling of these RTKs enhances the ability of T-cells (a type of white blood cell that is of key importance to the immune system) to recognize and eliminate tumor cells and modifies the tumor immune environment to enable a more productive immune response. The ability of sitravatinib to enhance the activity of immune checkpoint inhibitors was demonstrated in nonclinical cancer models.

#### Program Update

We are evaluating the potential of sitravatinib to enhance and expand the clinical efficacy of immune checkpoint inhibitors in a Phase 2 clinical trial in combination with nivolumab (Opdivo®), Bristol Myers Squibb's anti-PD-1 inhibitor, in patients with non-small cell lung cancer ("NSCLC"). This clinical trial is designed to assess the potential of sitravatinib to inhibit several important immunosuppressive pathways that may be important in overcoming resistance to checkpoint inhibitor therapy. This clinical trial is enrolling patients who have relapsed after treatment with a checkpoint inhibitor, as well as checkpoint inhibitor-naïve patients. As previously reported at the IASLC 18th World Conference on Lung Cancer, as of August 10, 2017, three of 11 evaluable patients who had relapsed after previous checkpoint inhibitor treatment experienced a confirmed partial response and seven of the 11 evaluable patients were continuing to receive treatment in the clinical trial, with treatment duration ranging from four months to 10.5 months. Early safety data indicated an acceptable profile and manageable adverse events. Based on the data presented, the pre-defined criteria for expansion were met and the next stage will enroll a cumulative total of 34 patients. We expect to report further data on checkpoint inhibitor refractory patients from this clinical trial in mid-2018. Based on sitravatinib's mechanism of action, we believe that there are multiple additional tumor types that would be attractive targets for further clinical development in combination with immune checkpoint inhibitors, including renal cell, liver and bladder cancers.

#### Sitravatinib as a Targeted Agent

##### Background

Dysregulation of RTKs through genetic alteration or uncontrolled expression is associated with multiple processes relating to human cancer, including tumor growth and metastatic progression, as well as tumor angiogenesis. Sitravatinib potentially inhibits a subset of these RTKs, including the TAM and split family receptors and RET, which are key regulators of signaling pathways that direct cell growth and survival. Through the genetic selection of patients with tumors that are driven by one or more of these dysregulated RTKs, there are multiple opportunities to develop sitravatinib as a targeted agent.

##### Program update

We are evaluating sitravatinib as a single agent in a Phase 1b expansion clinical trial (the "CITRINE Trial"), which is testing sitravatinib for the treatment of patients whose tumors harbor CBL, amplification of a genomic region located on chromosome 4q12 ("CHR4q12") and RET genetic alterations in NSCLC and other tumor types. Patients who have mutations in CBL, CHR4q12, or rearrangement of the RET gene are most likely to respond to sitravatinib because each of these mutations dysregulate different sitravatinib RTK targets. These mutations are estimated to be present in a total of 5.5% of all NSCLC patients. We reported early data from this clinical trial in January 2017 showing that as of December 9, 2016, of the four evaluable patients with RET genetic alterations at the time, there was one patient with stable disease, one unconfirmed partial response and one confirmed partial response. In September 2017, we presented a case study at the IASLC 2017 Chicago Multidisciplinary Symposium in Thoracic Oncology of an NSCLC patient with a CBL inactivating mutation. The case was the first evaluable NSCLC patient harboring a CBL mutation treated in the ongoing Phase 1b study of sitravatinib as a single agent. The confirmed partial response is the first example of clinical activity for sitravatinib in a patient with a CBL mutation. Inactivating mutations in CBL occur in approximately 1.5% of NSCLC patients and currently represent an unmet medical need. To date, sitravatinib safety data indicate an acceptable profile and manageable adverse events. We expect to provide an update on this clinical trial in mid-2018.

Collaboration with BeiGene, Ltd. to Develop and Commercialize Sitravatinib in Certain Asian Territories

In January 2018, we entered into a Collaboration and License Agreement (the “BeiGene Agreement”) with BeiGene, Ltd. (“BeiGene”), pursuant to which Mirati and BeiGene agreed to collaboratively develop sitravatinib in Asia (excluding Japan and certain other countries), Australia and New Zealand (the “Licensed Territory”). Under the BeiGene Agreement, Mirati granted BeiGene an exclusive license to develop, manufacture and commercialize sitravatinib in the Licensed Territory, with Mirati retaining exclusive rights for the development, manufacturing and commercialization of sitravatinib outside the Licensed Territory.

Under the BeiGene Agreement, BeiGene agreed to pay to Mirati an upfront fee of \$10.0 million. BeiGene is also required to make milestone payments to Mirati of up to an aggregate of \$123.0 million upon the first achievement of specified clinical, regulatory and sales milestones. Additionally, BeiGene is obligated to pay to Mirati royalties at tiered percentage rates ranging from mid-single digits to twenty percent on annual net sales of licensed products in the Licensed Territory, subject to reduction under specified circumstances.



## Mocetinostat

### Background

Mocetinostat is an oral, Class 1 selective histone deacetylase (“HDAC”) inhibitor. Mocetinostat acts through epigenetic mechanisms and has demonstrated in preclinical studies the ability to block the effects of immune suppressive cells that counter the immune system’s ability to fight tumors and reduce the effectiveness of treatment with checkpoint inhibitors. Epigenetics is the regulation of gene expression and resulting cellular phenotypes through mechanisms other than primary DNA sequence alterations. The epigenetic regulation of gene expression involves the regulation of DNA methylation and modification of certain histones via modulation of acetylation or methylation of specific amino acid residues. Epigenetic pathways can become dysregulated during cancer progression through a variety of mechanisms, including the genetic alteration of molecules that participate in DNA methylation and histone modification. The epigenetic mechanisms of HDAC inhibitors like mocetinostat have demonstrated efficacy in hematologic malignancies and have been approved as single agents and may be complementary with other epigenetic mechanisms.

### Program update

We are evaluating mocetinostat in a Phase 2 clinical trial in combination with durvalumab (IMFINZI™), MedImmune Limited’s anti-PD-L1 inhibitor, for the treatment of patients with NSCLC. The clinical trial is enrolling patients who have relapsed after treatment with a checkpoint inhibitor, as well as checkpoint inhibitor-naïve patients. Patients who have relapsed after previous treatment with a checkpoint inhibitor are stratified into two cohorts based upon their best response to prior checkpoint therapy. Stage 1 of the trial is currently enrolling nine patients in each cohort; one cohort has already met the prespecified criteria for expansion into stage 2 with at least one confirmed partial response. We expect to provide an update on stage 1 of this trial in mid-2018.

In October 2017, we announced that mocetinostat has been included in the SU2C Catalyst® program, a cutting-edge research initiative led by Stand up to Cancer (“SU2C”) designed to bring innovative cancer treatments to patients quickly through novel collaborations between industry and academia. The Phase 1/1b clinical trial, sponsored by Memorial Sloan Kettering Cancer Center, is designed to evaluate the potential of epigenetic agents to improve patient responses to immunotherapy in NSCLC which will combine mocetinostat, guadecitabine, a DNA methyltransferase inhibitor from Astex Pharmaceuticals, Inc., and pembrolizumab, a PD-1 checkpoint inhibitor from Merck & Co., Inc. (known as MSD outside the United States and Canada). The clinical trial enrolled its first patient in August 2017.

## KRAS G12C Inhibitor Program

### Background

The RAS family of genes is the most commonly mutated oncogene and mutations in this gene family comprise up to 25% of all human cancers. Among the RAS family members, mutations most frequently occur in KRAS (85% of all RAS family mutations). Tumors characterized by KRAS mutations are commonly associated with poor prognosis and resistance to therapy. Nonclinical studies have demonstrated that cancer cells exhibiting KRAS mutations are highly dependent on KRAS function for cell growth and survival. Historically, KRAS has been extremely difficult to directly inhibit due to the absence of a tractable small molecule drug binding site. Recent findings have indicated that the KRAS G12C mutant variant may be targeted by irreversible small molecules that bind to a novel drug pocket and inhibit the function of KRAS. KRAS G12C driver mutations occur in approximately 14% of NSCLC adenocarcinoma patients, 5% of colorectal cancer patients and smaller percentages in other tumor types. Collectively, these patients have few treatment options. We have selected a KRAS G12C mutant-selective clinical lead compound for advancement to an Investigational New Drug (“IND”) application. The clinical lead compound is an orally-available small molecule inhibitor of KRAS G12C with potency of approximately 10 nM (cellular IC<sub>50</sub>) and selectivity of greater than 1,000-fold for target inhibition in tumor cells harboring KRAS G12C mutations compared with cells exhibiting non-mutated forms of KRAS. In addition, the clinical lead compound demonstrated complete regression of KRAS G12C-positive tumors implanted in mice at well-tolerated dose levels.

### Program Update

In November 2017, we announced that IND-enabling preclinical studies were underway, and an IND submission for the KRAS G12C program is expected by the fourth quarter of 2018, with early clinical proof-of-concept data anticipated in 2019. The KRAS program emerged from a joint discovery collaboration with Array BioPharma, Inc.,

and Mirati has an exclusive license to further develop and commercialize products emerging from the collaboration.  
Glesatinib

In November 2017, we announced that, in light of superior investment opportunities in our pipeline, we suspended further investment in glesatinib and will pursue opportunities to partner the program.

## Market and Competition

### Market

#### Non-Small Cell Lung Cancer

The National Cancer Institute estimates that in 2017, approximately 223,000 patients in the United States ("U.S.") were diagnosed with lung cancer and 156,000 died due to the disease. Lung cancer represents over 13% of all new cancer cases in the U.S., and 26% of all cancer deaths. Approximately 85% of lung cancers are NSCLC. The five-year survival rate for lung cancer patients is 18%, indicating a significant need for novel therapies to extend overall survival in this patient population.

#### Immuno-oncology Combinations

The prognosis for advanced NSCLC is poor, and the primary objective of treating late-stage disease is to prolong overall survival, delay disease progression and control symptoms. The treatment algorithm for advanced NSCLC has changed significantly following recent approvals and label expansions of immuno-oncology agents, specifically immune checkpoint inhibitors. In 2015, the U.S. Food and Drug Administration ("FDA") approved Opdivo®, an anti-PD-1 monoclonal antibody, and the first immuno-oncology agent approved for the treatment of squamous NSCLC. The approval of Opdivo® in NSCLC was subsequently followed by FDA approval of two additional immuno-oncology agents in NSCLC, Keytruda® and Tecentriq®. These three agents, approved for indications including NSCLC, accounted for over \$6 billion in global sales in 2016. The immune checkpoint inhibitor market is projected to grow to \$46 billion by 2023<sup>(1)</sup>.

Despite the advances in patient outcomes demonstrated by approved immuno-oncology therapies in NSCLC, a significant patient need remains. The percentage of patients who respond to approved immuno-oncology treatments is quite low, and of the patients that respond, the majority will still experience disease progression. We believe that combinations of checkpoint inhibitors with other agents like sitravatinib and mocetinostat have the potential to improve efficacy outcomes and overcome resistance to checkpoint inhibitor therapy through complementary mechanisms.

#### Targeted Agents

NSCLC represents a heterogeneous patient population with diverse tumor histology and underlying genomic aberrations. Genetic alterations consisting of CBL mutations, CHR4q12 amplifications and RET gene rearrangements account for up to 5.5% of NSCLC patient cases annually in the U.S. KRAS G12C driver mutations occur in approximately 14% of NSCLC adenocarcinoma patients and 5% of colorectal cancer patients.

The clinical and commercial success of leading targeted agents across multiple indications, including NSCLC, demonstrates the potential of new targeted treatments for cancer. The following table lists global sales figures for selected small molecule kinase inhibitors in NSCLC and other indications.

#### 2016 Worldwide Retail Sales Figures of Selected Small Molecule Kinase Inhibitors

| Brand Name          | 2016<br>Worldwide<br>Sales <sup>(2)</sup> (in<br>millions) |
|---------------------|------------------------------------------------------------|
| Tarceva             | \$ 1,039                                                   |
| Tafinlar + Mekinist | \$ 672                                                     |
| Xalkori             | \$ 561                                                     |
| Tagrisso            | \$ 423                                                     |
| Alecensa            | \$ 186                                                     |

<sup>(1)</sup>Decision Resources.

<sup>(2)</sup>Source: Evaluate Pharma.



## Competition

### Sitravatinib in Combination with Immune Checkpoint Inhibitors

There are several immune checkpoint inhibitors currently approved for use as single agents to treat multiple tumor types, including NSCLC. To augment the efficacy of these agents, combination studies are being conducted with a variety of potentially synergistic mechanisms, including inhibitors of CTLA-4, IDO1, LAG3, and CSF-1R. Most of these combination studies are being conducted in patients who are naïve to immune checkpoint inhibitor therapy. A competitor whose agent is being evaluated in combination with checkpoint inhibitors in NSCLC patients that are naïve to immune checkpoint inhibitor therapy is Nektar Therapeutics (CD-122 agonist). Competitors whose agents are being evaluated in combination with checkpoint inhibitors in NSCLC patients who failed previous immune checkpoint inhibitor therapy include Corvus Pharmaceuticals, Inc. (Adenosine A2Ar inhibitor), Bristol-Myers Squibb (GITR inhibitor and LAG3 inhibitor) and Syndax, Inc. (HDAC inhibitor). Direct mechanistic competitors to sitravatinib in immunotherapy include Cabometyx (Exelixis, Inc.), an anti-VEGF agent that also inhibits MET and other RTKs. We expect that additional agents and mechanisms will be evaluated in immunotherapy combinations, and we will continue to monitor these competitors in relation to our own immunotherapy combination programs.

### Sitravatinib as a Targeted Agent

A large number of multi-targeted kinase inhibitors are currently commercially available or in clinical trials, with many more in the early research stage. Biotechnology and pharmaceutical companies are also developing monoclonal antibodies to inhibit kinase targets and their ligands.

Companies with RET inhibitors believed to be in late preclinical or clinical development include, but are not limited to, Blueprint Medicines, Inc., Ignyta, Inc., and Loxo Oncology, Inc. For CBL and CHRq12 alterations, we are not aware of any other companies with programs that specifically target these patient populations.

### Mocetinostat

We believe that a key differentiating feature of mocetinostat is its spectrum of activity covering only isoforms 1, 2, 3 and 11, which are the most relevant HDAC isoforms in human cancers. Other companies that are developing spectrum-selective HDAC inhibitors include, but are not limited to, Acetylon Pharmaceuticals, Inc., Chroma Therapeutics Ltd., Huya Bioscience International, Shenzhen Chipscreen Biosciences Ltd. and Syndax Pharmaceuticals Inc.

Companies with Pan-HDAC inhibitors, which are HDAC inhibitors that have an effect across a broader range of HDAC isoforms and are therefore not as selective as molecules like mocetinostat, include but are not limited to: Celgene Corporation, Curis Inc., MEI Pharma Inc., Merck & Co Inc., Novartis, Pharmacyclics Inc. and others. We expect that these and other companies may continue to pursue research and development in relation to HDAC inhibitors. We continue to monitor these and other companies in order to be aware of any third-party products and/or intellectual property rights relevant to our products.

### KRAS G12C

We are aware of one company with a competing direct KRAS G12C inhibitor program: Araxes Pharma, which partnered its KRAS G12C program with Janssen Biotech in 2013. It is likely that other companies are also researching KRAS G12C inhibitors. In November 2017, our first patent application covering our KRAS G12C inhibitor program was published. Based upon this and other publicly available information, we believe that our program is the most advanced. We will continue to monitor scientific and patent publications for the emergence of other potential competitors.

### Oncology

In addition to companies that have kinase inhibitors or HDAC inhibitors addressing our targets of interest, our competition also includes hundreds of private and publicly traded companies that operate in the area of oncology but have therapeutics with different mechanisms of action. The oncology market in general is highly competitive, with over 1,000 molecules currently in clinical development. Other important competitors, in addition to those mentioned above, are small and large biotechnology companies, including, but not limited to, Amgen, Inc. and Gilead Sciences Inc., and specialty and regional pharmaceutical companies and multinational pharmaceutical companies, including but not limited to Astellas Pharma Inc., Bayer-Schering Pharmaceutical, Boehringer Ingelheim AG, Bristol-Myers Squibb, Eisai Co. Ltd., Eli Lilly and Company, F. Hoffmann- LaRoche Ltd., GlaxoSmithKline, Johnson & Johnson,

Merck KGaA, Novartis AG, Taiho and Takeda Pharmaceutical Co.

Many companies have filed, and continue to file, patent applications which may or could affect our program if and when they issue, either because they protect a product that may compete with our product candidates, or because they protect intellectual property rights that are necessary for us to develop and commercialize our product candidates. These companies include, but are not limited to: Bristol-Myers Squibb, Exelixis, GlaxoSmithKline, Novartis and Pfizer. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, we expect that these and other companies will continue to publish and file patent applications in this space in the future, as well as pursuing research and development programs in this area. We continue

to monitor these and other companies in order to be aware of any third-party products and/or intellectual property rights relevant to our product candidates.

#### Intellectual Property

Our goal is to obtain, maintain and enforce patent protection wherever appropriate for our product candidates, formulations, processes, methods and any other proprietary technologies both in the United States and in other countries. We typically file for patents in the United States with counterparts in certain countries in Europe and certain key market countries in the rest of the world, thereby covering the major pharmaceutical markets. As of December 31, 2017, we own or co-own U.S. patents and patent applications and their foreign counterparts, including 28 issued U.S. patents as reflected in the following table:

| Description                                       | Granted | Pending | Expiration |
|---------------------------------------------------|---------|---------|------------|
| Sitravatinib and other kinase inhibitor compounds | 11      | 0       | 2026-2029  |
| Mocetinostat and other HDAC inhibitors            | 17      | 0       | 2022-2035  |
| KRAS inhibitors                                   | 0       | 3       | 2036-2037  |
| Total                                             | 28      | 3       |            |

#### Manufacturing

We do not own or operate manufacturing facilities for the production of any of our product candidates, nor do we plan to develop our own manufacturing operations in the foreseeable future. We currently depend on third-party contract manufacturers for all of our required raw materials and finished products for our preclinical and clinical trials.

Manufacturers of our products are required to comply with applicable FDA manufacturing requirements contained in the FDA's Current Good Manufacturing Practices ("cGMP") regulations. cGMP regulations require, among other things, quality control and quality assurance as well as corresponding maintenance of records and documentation.

Pharmaceutical product manufacturers and other entities involved in the manufacture and distribution of approved pharmaceutical products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer or holder of an approved new drug applications ("NDA"), including withdrawal of the product from the market. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented.

#### Government Regulation

##### The Regulatory Process for Drug Development

The production and manufacture of our product candidates and our research and development activities are subject to regulation by various governmental authorities around the world. In the United States, drug products are subject to regulation by the FDA. There are other comparable agencies in Europe and other parts of the world. Regulations govern, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of products. Applicable legislation requires licensing of manufacturing and contract research facilities, carefully controlled research and testing of products, governmental review and/or approval of results prior to marketing therapeutic products. Additionally, adherence to good laboratory practices ("GLP") and good clinical practices ("GCP") during nonclinical and clinical testing and cGMP during production is required.

##### U.S. Pharmaceutical Product Development Process

In the United States, the FDA regulates pharmaceutical products under the Federal Food, Drug and Cosmetic Act and implementing regulations. Pharmaceutical products are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product

recalls, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a pharmaceutical product may be marketed in the United States generally includes the following:



• completion of preclinical laboratory tests, animal studies and formulation studies in compliance with good laboratory practice, or GLP, regulations;

• submission to the FDA of an IND, which must become effective before human clinical trials may begin;

• performance of adequate and well-controlled human clinical trials in accordance with GCP standards and regulations to establish the safety and efficacy of the proposed drug for each indication;

• submission to the FDA of an NDA;

• satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; and

• FDA review and approval of the NDA.

The FDA monitors the progress of trials conducted in the U.S. under an IND and may, at its discretion, re-evaluate, alter, suspend or terminate testing based on the data accumulated to that point and the FDA's risk/benefit assessment with regard to the patients enrolled in the trial. The FDA may also place a hold on one or more clinical trials conducted under an IND for a drug if it deems warranted. Furthermore, even after regulatory approval of an NDA is obtained, under certain circumstances, such as later discovery of previously unknown problems, the FDA can withdraw approval or subject the drug to additional restrictions.

**Preclinical Studies:** Preclinical studies include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to evaluate efficacy and activity, toxic effects, pharmacokinetics and metabolism of the pharmaceutical product candidate and to provide evidence of the safety, bioavailability and activity of the pharmaceutical product candidate in animals. Most of these studies must be performed according to GLP.

**Clinical Trials:** Clinical trials involve the administration of the pharmaceutical product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by the sponsor. Clinical trials must be conducted in accordance with the FDA's GCP requirements. Further, each clinical trial must be reviewed and approved by an independent institutional review board ("IRB"), or ethics committee at or servicing each institution at which the clinical trial will be conducted. An IRB or ethics committee is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB or ethics committee also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until completed.

Clinical trials in the U.S. typically are conducted in sequential phases: Phases 1, 2, 3 and post-approval clinical trials, sometimes referred to as Phase 4 clinical trials. The phases may overlap. The FDA may require that we suspend clinical trials at any time on various grounds.

**Phase 1 Clinical Trials:** Phase 1 clinical trials are generally conducted on a small number of healthy human subjects to evaluate the drug's activity, schedule and dose, absorption, metabolism, distribution, excretion and other drug effects. However, in the case of life-threatening diseases, such as cancer, the initial Phase 1 testing may be done in patients with the disease. These trials typically take longer to complete and may provide insights into drug activity. Follow-on Phase 1b clinical trials may also evaluate efficacy with respect to trial participants.

**Phase 2 Clinical Trials:** Phase 2 clinical trials are carried out on a relatively small number of patients (generally up to several hundred) in a specific indication. The pharmaceutical product is evaluated to preliminarily assess efficacy, to identify possible adverse effects and safety risks, and to determine optimal dose, regimens, pharmacokinetics, pharmacodynamics and dose response relationships. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning Phase 3 clinical trials.

**Phase 3 Clinical Trials:** Phase 3 clinical trials involve tests on a much larger population of patients (several hundred to several thousand patients) suffering from the targeted condition or disease. These trials are undertaken to confirm proof of concept and further evaluate dosage, clinical efficacy and safety and are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. Generally, two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA or foreign authorities for approval of marketing applications.

**Post-Approval Clinical Trials:** Phase 4 clinical trials or other post-approval commitments may be conducted after initial marketing approval. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and may be required by the FDA as a condition of approval.

Progress reports detailing the results of the clinical trial must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or for any finding from tests in laboratory animals that suggests a significant risk for human subjects. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or, if used, its data safety and monitoring board may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB or ethics committee can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's or ethics committee's requirements or if the pharmaceutical product has been associated with unexpected serious harm to patients.

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

#### U.S. Pharmaceutical Review and Approval Process

Upon completion of pivotal Phase 3 clinical studies, the sponsor assembles all the product development, preclinical and clinical data along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the pharmaceutical product, proposed labeling and other relevant information, and submits it to the FDA as part of an NDA. If accepted by the FDA as substantially complete to permit substantive review, the submission or application is then reviewed for approval to market the product. This process takes eight months to one year to complete, but in some cases may take longer. At the end of the review period the FDA may issue a Complete Response Letter, refusing to approve an NDA if the applicable regulatory criteria are not satisfied or requiring additional clinical data or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling.

#### Accelerated Approval

Accelerated Approval is a program that is intended to make promising products for life threatening diseases available on the basis of evidence of effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Approvals of this kind typically include requirements for appropriate post-approval Phase 4 clinical trials to validate the surrogate endpoint or otherwise confirm the effect of the clinical endpoint.

#### Post-Approval Requirements

Any pharmaceutical products for which we receive FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, complying with certain electronic records and signature requirements and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, promoting pharmaceutical products for uses or in patient populations that are not described in the pharmaceutical product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities and promotional activities involving the internet. Failure to comply with FDA requirements can have negative consequences, including adverse publicity, enforcement letters from the FDA, mandated corrective advertising or communications with doctors and civil or criminal penalties.

The FDA also may require post-marketing testing, known as Phase 4 testing, risk evaluation and mitigation strategies and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict

the distribution or use of the product.

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#### FDA Regulation of Companion Diagnostics

As part of our clinical development plans, we are exploring the use of companion diagnostics to identify patients most likely to respond to our product candidates. Companion diagnostics are classified as medical devices under the Federal Food, Drug, and Cosmetic Act in the United States. In the United States, the FDA regulates the medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, reporting, recordkeeping, advertising and promotion, export and import, sales and distribution, and post-market surveillance of medical devices. Unless an exemption applies, companion diagnostics require marketing clearance or approval from the FDA prior to commercial distribution. The two primary types of FDA marketing authorization applicable to a medical device are premarket notification, also called 510(k) clearance, and premarket approval ("PMA").

The FDA previously has required in vitro companion diagnostics intended to select the patients who will respond to the cancer treatment to obtain a PMA simultaneously with approval of the drug. Based on the draft guidance, and the FDA's past treatment of companion diagnostics, we believe that the FDA will require a PMA for one or more companion diagnostics to identify patient populations suitable for our product candidates. The review of these companion diagnostics in conjunction with the review of our product candidates involves coordination of review by the FDA's Center for Drug Evaluation and Research and by the FDA's Center for Devices and Radiological Health.

#### Pharmaceutical Coverage, Pricing and Reimbursement

In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and adequate reimbursement from third-party payors, including government authorities, managed care providers, private health insurers and other organizations. In the United States, private health insurers and other third-party payors often provide reimbursement for products and services based on the level at which the government (through the Medicare or Medicaid programs) provides reimbursement for such products and services. There is no uniform coverage and reimbursement policy among third-party payors in the United States; however, private third-party payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Third-party payors are increasingly examining the medical necessity and cost-effectiveness of medical products and services in addition to their safety and efficacy and, accordingly, significant uncertainty exists as to the coverage and reimbursement status of newly approved therapeutics. In particular, in the United States, the European Union and other potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which often has resulted in average selling prices that are lower than they would otherwise be. Recently, Congress and the Trump Administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Further, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and utilization, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general. As a result, coverage and adequate third-party reimbursement may not be available for our product candidates to enable us to realize an appropriate return on our investment in research and product development.

The market for our product candidates for which we may receive regulatory approval will depend significantly on access to third-party payors' drug formularies, or lists of medications for which third-party payors provide coverage and reimbursement. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or may otherwise restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available. In addition, because each third-party payor individually approves coverage and reimbursement levels, obtaining coverage and adequate reimbursement is a time-consuming and costly process. We would be required to provide scientific and clinical support for the use of any product to each third-party payor separately with no assurance that approval would be obtained, and we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products. This process could delay

the market acceptance of any of our product candidates for which we may receive approval and could have a negative effect on our future revenue and operating results. We cannot be certain that our product candidates will be considered cost-effective. If we are unable to obtain coverage and adequate payment levels for our product candidates from third-party payors, physicians may limit how much or under what circumstances they will prescribe or administer them and patients may decline to purchase them. This in turn could affect our ability to successfully commercialize our products and impact our profitability, results of operations, financial condition, and future success.

#### Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product,

we would need to obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

#### Other Healthcare Laws and Compliance Requirements

Several other types of state and federal laws restrict certain marketing practices in the pharmaceutical industry. These laws, which generally will not be applicable to us or our product candidates unless and until we obtain FDA marketing approval for any of our product candidates, include state and federal anti-kickback, fraud and abuse, false claims, physician payment, sunshine, patient protection and affordable care, privacy and security laws and regulations regarding providing drug samples. . In addition, there have been a number of substantial legislative and regulatory changes to the way healthcare is financed and paid for by both governmental and private insurers, including the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the “ACA”). There has been, and continues to be, significant developments in, and continued legislative activity around the ACA and related laws, which could result in the repeal or repeal and replacement of all or certain elements of the ACA.

We may in the future be subject to the Foreign Corrupt Practices Act of 1997 (“FCPA”). The FCPA and other similar anti-bribery laws in other jurisdictions, such as the U.K. Bribery Act, generally prohibit companies and their intermediaries from providing money or anything of value to officials of foreign governments, foreign political parties, or international organizations with the intent to obtain or retain business or seek a business advantage. A determination that our operations or activities are not, or were not, in compliance with United States or foreign laws or regulations could result in the imposition of substantial fines, interruptions of business, loss of supplier, vendor or other third-party relationships, termination of necessary licenses and permits and other legal or equitable sanctions.

#### Other Laws

In addition to the above, we are subject to a variety of financial disclosure and securities trading regulations as a public company in the U.S., including laws relating to the oversight activities of the SEC and the regulations of The NASDAQ Stock Exchange, on which our shares are traded. We are also subject to various laws, regulations and recommendations relating to safe working conditions, laboratory practices and the experimental use of animals.

#### Research and Development Expenses

Our research and development expenses were \$58.1 million, \$68.5 million, and \$49.0 million for the years ended December 31, 2017, 2016 and 2015, respectively.

#### Employees

As of December 31, 2017, we had 51 employees located in our offices in San Diego. 34 employees are engaged in research and development activities and 17 are in general and administrative functions.

#### Corporate Information

We were incorporated under the laws of the State of Delaware on April 29, 2013 as Mirati Therapeutics, Inc. Our website address is [www.mirati.com](http://www.mirati.com). Our website and the information contained on, or that can be accessed through, the website will not be deemed to be incorporated by reference in, and are not considered part of, this Annual Report on Form 10 K. Our Annual Reports on Form 10 K, Quarterly Reports on Form 10 Q, Current Reports on Form 8 K and amendments to reports filed or furnished pursuant to Section 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended, are available free of charge on the Investors portion of our web site at [www.mirati.com](http://www.mirati.com) as soon as reasonably practical after we electronically file such material with, or furnish it to, the Securities and Exchange Commission (“SEC”).

Item 1A. Risk Factors

**RISK FACTORS**

Except for the historical information contained herein, this Annual Report on Form 10-K and the information incorporated by reference herein contains forward-looking statements that involve risks and uncertainties. These statements include projections about our accounting and finances, plans and objectives for the future, future operating and economic performance and other statements regarding future performance. These statements are not guarantees of future performance or events. Our actual results may differ materially from those discussed here. Factors that could cause or contribute to such differences are described in the following section as well as those discussed in Part II, Item 7 entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations," and elsewhere throughout this report and in any other documents incorporated by reference herein. There may be additional risks that we do not presently know of or that we currently believe are immaterial which could also impair our business and financial position. We disclaim any obligation to update any forward-looking statement.

**Risks Relating to Our Financial Position and Capital Requirements**

We will require additional financing and may be unable to raise sufficient capital, which could lead us to delay, reduce or abandon development programs or commercialization.

Our operations have consumed substantial amounts of cash since inception. Our research and development expenses were \$58.1 million, \$68.5 million, and \$49.0 million for the years ended December 31, 2017, 2016 and 2015, respectively. We will require substantial additional capital to pursue additional clinical development for our lead clinical programs, including conducting late-stage clinical trials, manufacturing clinical supplies and potentially developing other assets in our pipeline, and, if we are successful, to commercialize any of our current product candidates. If the U.S. Food and Drug Administration ("FDA") or any foreign regulatory agency, such as the European Medicines Agency ("EMA") requires that we perform studies or trials in addition to those that we currently anticipate with respect to the development of our product candidates, or repeat studies or trials, our expenses would further increase beyond what we currently expect. We may not be able to adequately finance our development programs, which could limit our ability to move our programs forward in a timely and satisfactory manner or require us to abandon the programs, any of which would harm our business, financial condition and results of operations. Because successful development of our product candidates is uncertain, we are unable to estimate the actual funds we will require to complete research and development and commercialize our product candidates.

If we are unable to obtain funding from equity offerings or debt financings on a timely basis, we may be required to (1) seek additional collaborators for one or more of our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available; (2) relinquish or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves; or (3) significantly curtail one or more of our research or development programs or cease operations altogether.

We are a clinical-stage company with no approved products and no historical product revenue. Consequently, we expect that our financial and operating results will vary significantly from period to period.

We are a clinical-stage company that has incurred losses since its inception and expect to continue to incur substantial losses in the foreseeable future. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty.

Our actual financial condition and operating results have varied significantly in the past and are expected to continue to fluctuate significantly from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond our control. Factors relating to our business that may contribute to these fluctuations include:



the success of our clinical trials through all phases of clinical development;

delays in the commencement, enrollment and timing of clinical trials;

our ability to secure and maintain collaborations, licensing or other arrangements for the future development and/or commercialization of our product candidates, as well as the terms of those arrangements;

our ability to obtain, as well as the timeliness of obtaining, additional funding to develop our product candidates;

the results of clinical trials or marketing applications for product candidates that may compete with our product candidates;

- competition from existing products or new products that may receive marketing approval;
- potential side effects of our product candidates that could delay or prevent approval or cause an approved drug to be taken off the market;
- any delays in regulatory review and approval of our clinical development plans or product candidates;
- our ability to identify and develop additional product candidates;
- the ability of patients or healthcare providers to obtain coverage or sufficient reimbursement for our products;
- our ability, and the ability of third parties such as Clinical Research Organizations ("CROs") to adhere to clinical study and other regulatory requirements;
- the ability of third-party manufacturers to manufacture our product candidates and key ingredients needed to conduct clinical trials and, if approved, successfully commercialize our products;
- the costs to us, and our ability as well as the ability of any third-party collaborators, to obtain, maintain and protect our intellectual property rights;
- costs related to and outcomes of potential intellectual property litigation;
- our ability to adequately support future growth;
- our ability to attract and retain key personnel to manage our business effectively; and
- our ability to build our finance infrastructure and, to the extent required, improve our accounting systems and controls.

Accordingly, the likelihood of our success must be evaluated in light of many potential challenges and variables associated with a clinical-stage company, many of which are outside of our control, and past operating or financial results should not be relied on as an indication of future results. Fluctuations in our operating and financial results could cause our share price to decline. It is possible that in some future periods, our operating results will be above or below the expectations of securities analysts or investors, which could also cause our share price to decline.

We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future. We have never generated any revenue from product sales and may never be profitable.

We have derived limited revenue from our research, collaboration and licensing agreements which has not been sufficient to cover the substantial expenses we have incurred in our efforts to develop our product candidates. Consequently, we have accumulated net losses since inception in 1995. Our net loss for the years ended December 31, 2017, 2016, and 2015 were \$70.4 million, \$83.1 million, and \$64.5 million respectively. As of December 31, 2017, we had an accumulated deficit of \$460.6 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. Such losses are expected to increase in the future as we continue the development of our product candidates and seek regulatory approval and commercialization for our product candidates. We are unable to predict the extent of any future losses or when we will become profitable, if ever. Even if we do achieve profitability, we may not be able to sustain or increase profitability on an ongoing basis.

We do not anticipate generating revenue from sales of products for the foreseeable future, if ever. If any of our product candidates fail in clinical trials or do not gain regulatory approval, or if any of our product candidates, if approved, fail to achieve market acceptance, we may never become profitable. If one or more of our product candidates is approved for commercial sale and we retain commercial rights, we anticipate incurring significant costs associated with commercializing any such approved product candidate. Therefore, even if we are able to generate revenue from the sale of any approved product, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing development and clinical trial programs for our product candidates;
- maintaining existing collaboration and licensing agreements and entering into additional ones;

- seeking and obtaining marketing approvals for any product candidates that successfully complete clinical trials;
- establishing and maintaining supply and manufacturing relationships with third parties;
- successfully commercializing any product candidates for which marketing approval is obtained; and
- successfully establishing a sales force and marketing and distribution infrastructure.

Raising additional funds through debt or equity financing will be dilutive and raising funds through licensing agreements may be dilutive, restrict operations or relinquish proprietary rights.

To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Existing stockholders may not agree with our financing plans or the terms of such financings. Moreover, the incurrence of debt financing could result in a substantial portion of our operating cash flow being dedicated to the payment of principal and interest on such indebtedness and could impose restrictions on our operations. In addition, if we raise additional funds through future collaboration and licensing arrangements, it may be necessary to relinquish potentially valuable rights to our products or proprietary technologies, or to grant licenses on terms that are not favorable to us. Additional funding may not be available to us on acceptable terms, or at all.

As a public company in the United States, we incur significant legal and financial compliance costs and we are subject to the Sarbanes-Oxley Act. We can provide no assurance that we will, at all times, in the future be able to report that our internal controls over financial reporting are effective.

Companies that file reports with the Securities and Exchange Commission ("SEC"), including us, are subject to the requirements of Section 404 of the Sarbanes-Oxley Act of 2002. Section 404 requires management to establish and maintain a system of internal control over financial reporting, and annual reports on Form 10-K filed under the Securities Exchange Act of 1934, as amended ("the Exchange Act"), must contain a report from management assessing the effectiveness of a company's internal control over financial reporting. Ensuring that we have adequate internal financial and accounting controls and procedures in place to produce accurate financial statements on a timely basis remains a costly and time-consuming effort that needs to be re-evaluated frequently. Failure on our part to have effective internal financial and accounting controls would cause our financial reporting to be unreliable, could have a material adverse effect on our business, operating results, and financial condition, and could cause our stock price to decline as a result.

As an "emerging growth company" (as defined in the JOBS Act), we are not required to comply with Section 404(b) which requires attestation from our external auditors on our internal control over financial reporting. We are subject to Section 404(a), which requires management to provide a report regarding the effectiveness of internal controls. We are required to review all of our control processes to align them to the Section 404 requirements. Failure to provide assurance that our financial controls are effective could lead to lack of confidence by investors which could cause our stock price to decline. When we are no longer an "emerging growth company" (as defined in the Exchange Act or the Securities Act of 1933, as amended (the "Securities Act")), our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting. The rules governing the standards that must be met for management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation. To continue complying with the requirements of being a reporting company under the Exchange Act, we may need to further upgrade our systems, including information technology, implement additional financial and management controls, reporting systems and procedures, and hire additional accounting and finance staff.

In addition, our independent registered public accounting firm has never performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act because no such evaluation has been required. Had our independent registered public accounting firm performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act, significant deficiencies or material weaknesses may have been identified. If we identify any significant deficiencies or material weaknesses that may exist or are unable to successfully remediate any significant deficiency or material weakness in our internal control over financial reporting, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our stock price may decline as a result.

Furthermore, shareholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance

costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, any new regulations or disclosure obligations may increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

We are an emerging growth company and we cannot be certain if the reduced disclosure requirements applicable to emerging growth companies will make our common stock less attractive to investors.

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

For as long as we continue to be an emerging growth company, we intend to take advantage of certain other exemptions from various reporting requirements that are applicable to other public companies including, but not limited to, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory stockholder vote on executive compensation and any golden parachute payments not previously approved, exemption from the requirement of auditor attestation in the assessment of our internal control over financial reporting and exemption from any requirement that may be adopted by the Public Company Accounting Oversight Board. If we do continue to be an emerging growth company, the information that we provide stockholders may be different than what is available with respect to other public companies. We cannot predict if investors will find our common stock less attractive because we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less-active trading market for our common stock and our stock price may be more volatile.

We expect that we will remain an emerging growth company until December 31, 2018.

Decreased disclosures in our SEC filings due to our status as an emerging growth company may make it harder for investors to analyze our results of operations and financial prospects.

The timing of the milestone and royalty payments we are entitled to receive from BeiGene, Ltd. is uncertain and could adversely affect our cash flows and results of operations.

In January 2018 we entered into a collaboration and license agreement with BeiGene, Ltd. (“BeiGene”) (the “BeiGene Agreement”), pursuant to which we agreed to collaboratively develop sitravatinib in Asia (excluding Japan and certain other countries), Australia and New Zealand (the “BeiGene Territory”) and we granted BeiGene an exclusive license to develop, manufacture and commercialize sitravatinib in the BeiGene Territory. In addition to an up-front payment, we may be entitled to receive up to an additional \$123.0 million upon the achievement of certain milestones under the BeiGene Agreement. However, the receipt of these payments is inherently uncertain. The receipt of milestone payments under the BeiGene Agreement can have a significant impact on our cash flows and results of operations for the periods of time in which such payments are made. While receipt of milestone and royalty payments would result in significant income, the absence of collaboration revenues in subsequent quarters could result in significant reductions in net income and could cause our stock price to drop.

#### Risks Relating to Our Business and Industry

Our research and development programs and product candidates are at an early stage of development. As a result, we are unable to predict if or when we will successfully develop or commercialize our product candidates.

Our clinical-stage product candidates as well as our other pipeline assets are at an early stage of development and will require significant further investment and regulatory approvals prior to commercialization. We currently have no product candidates beyond Phase 2 clinical trials. Mocetinostat is currently in a Phase 2 combination clinical trial, sitravatinib is in Phase 1b single agent and Phase 2 combination clinical trials and we have a KRAS inhibitor preclinical program. Each of our product candidates will require the selection of suitable patients for our clinical trials and additional clinical development, management of clinical, preclinical and manufacturing activities, obtaining regulatory approval, obtaining manufacturing supply, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenues from product sales. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. The treatment of cancer is a rapidly evolving field and will continue to evolve. By such time, if ever, as we may receive necessary regulatory approvals for our product candidates, the standard of care for the treatment of cancers may have evolved such that it would be necessary to modify our plans for full approval and commercial acceptance of our products may be limited by a change

in the standard of care. In addition, some of our product development programs contemplate the development of companion diagnostics. Companion diagnostics are subject to regulation as medical devices and we or our future collaborators may be required to obtain marketing approval for accompanying companion diagnostics before we may commercialize our product candidates.

Even if we obtain the required financing or establish a collaboration to enable us to conduct late-stage clinical development of our product candidates and pipeline assets, we cannot be certain that such clinical development would be successful, or that we will obtain regulatory approval or be able to successfully commercialize any of our product candidates and generate revenue. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and the clinical trial process may fail to demonstrate that our product candidates are safe and effective for their proposed uses. Any such failure could cause us to abandon further development of any one or more of our product candidates and may delay development of other product candidates. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. Any delay in, or termination of, our clinical trials will delay and possibly preclude the submission of any new drug applications ("NDAs") with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenue.

We have not previously submitted an NDA to the FDA, or similar drug approval filings to comparable foreign authorities, for any product candidate, and we cannot be certain that any of our product candidates will receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations. Even if we successfully obtain regulatory approvals to market one or more of our product candidates, our revenues will be dependent, in part, upon our or our collaborators' and future collaborators' ability to obtain regulatory approval for the companion diagnostics to be used with our product candidates, if required, and upon the size of the markets in the territories for which we gain regulatory approval and have commercial rights. If the markets for patient subsets that we are targeting are not as significant as we estimate, we may not generate significant revenues from sales of such products, if approved.

All of our product candidates are subject to extensive regulation, which can be costly and time consuming, cause delays or prevent approval of such product candidates for commercialization.

The clinical development of product candidates is subject to extensive regulation by the FDA in the United States and by comparable regulatory authorities in foreign markets. Product development is a very lengthy and expensive process, and its outcome is inherently uncertain. The product development timeline can vary significantly based upon the product candidate's novelty and complexity. Regulations are subject to change and regulatory agencies have significant discretion in the approval process.

Numerous statutes and regulations govern human testing and the manufacture and sale of human therapeutic products in the United States, Europe and other countries and regions where we intend to market our products. Such legislation and regulation bears upon, among other things, the approval of trial protocols and human testing, the approval of manufacturing facilities, safety of the product candidates, testing procedures and controlled research, review and approval of manufacturing, preclinical and clinical data prior to marketing approval including adherence to good manufacturing practices ("GMP") during production and storage as well as regulation of marketing activities including advertising and labeling.

In order to obtain regulatory approval for the commercial sale of any of our product candidates, we must demonstrate through preclinical studies and clinical trials that the potential product is safe and effective for use in humans for each target indication. The failure to adequately demonstrate the safety and efficacy of a product under development could delay or prevent regulatory approval of our product candidates.



No assurance can be given that current regulations relating to regulatory approval will not change or become more stringent in the United States or foreign markets. Regulatory agencies may also require that additional trials be run in order to provide additional information regarding the safety or efficacy of any drug candidates for which we seek regulatory approval. Moreover, any regulatory approval of a drug which is eventually obtained may entail limitations on the indicated uses for which that drug may be marketed. Furthermore, product approvals may be withdrawn or limited in some way if problems occur following initial marketing or if compliance with regulatory standards is not maintained. Regulatory agencies could become more risk averse to any side effects or set higher standards of safety and efficacy prior to reviewing or approving a product. This could result in a product not being approved. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

The failure to maintain the BeiGene Agreement or the failure of BeiGene to perform its obligations under the BeiGene Agreement, could negatively impact our business.

Pursuant to the terms of the BeiGene Agreement, we granted to BeiGene an exclusive license to develop, manufacture and commercialize sitravatinib in the BeiGene Territory. Consequently, our ability to generate any revenues from sitravatinib in the BeiGene Territory depends on our ability to maintain our collaboration with BeiGene. We have limited control over the amount and timing of resources that BeiGene will dedicate to these efforts.

We are subject to a number of other risks associated with our dependence on the BeiGene Agreement with respect to sitravatinib in the BeiGene Territory, including:

BeiGene may not comply with applicable regulatory guidelines with respect to developing, manufacturing or commercializing sitravatinib, which could adversely impact sales or future development of sitravatinib in the BeiGene Territory or elsewhere;

We and BeiGene could disagree as to future development plans and BeiGene may delay, fail to commence or stop future clinical trials or other development;

There may be disputes between us and BeiGene, including disagreements regarding the BeiGene Agreement, that may result in (1) the delay of or failure to achieve developmental, regulatory and commercial objectives that would result in milestone or royalty payments, (2) the delay or termination of any future development or commercialization of sitravatinib in the BeiGene Territory, and/or (3) costly litigation or arbitration that diverts our management's attention and resources;

BeiGene may not provide us with timely and accurate information regarding development, sales and marketing activities or supply forecasts, which could adversely impact our ability to comply with our obligations to BeiGene and manage our own inventory of sitravatinib, as well as our ability to generate accurate financial forecasts;

Business combinations or significant changes in BeiGene's business strategy may adversely affect BeiGene's ability or willingness to perform its obligations under the BeiGene Agreement; and

BeiGene may not properly defend our intellectual property rights, or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property rights or expose us to potential litigation.

The BeiGene Agreement is also subject to early termination, including through BeiGene's right to terminate without cause upon advance notice to us. If the agreement is terminated early, we may not be able to find another collaborator for the further development and commercialization of sitravatinib in the BeiGene Territory on acceptable terms, or at all, and we may be unable to pursue continued development and commercialization of sitravatinib in the BeiGene Territory on our own.

We may not be successful in establishing development and commercialization collaborations which could adversely affect, and potentially prohibit, our ability to develop our product candidates.

Because developing pharmaceutical products, conducting clinical trials, obtaining regulatory approval, establishing manufacturing capabilities and marketing approved products is expensive, we may seek to enter into additional collaborations with companies that have more resources and experience in order to continue to develop and commercialize our product candidates. We also may be required due to financial or scientific constraints to enter into additional collaboration agreements to research and/or to develop and commercialize our product candidates. The

establishment and realization of such collaborations may not be possible or may be problematic. There can be no assurance that we will be able to establish such additional collaborations on favorable terms, if at all, or that our current or future collaborative arrangements will be successful or maintained for any specific product candidate or indication. If we are unable to reach successful agreements with suitable collaboration partners for the ongoing development and commercialization of our product candidates, we may face increased costs, we may be forced to limit the scope and number of our product candidates we can commercially develop or the territories in which we commercialize such product candidates, and we may be unable to commercialize products or programs for which a suitable collaboration partner cannot be found. If we fail to achieve successful collaborations, our operating results and financial condition will be materially and adversely affected.

In addition, the terms of any collaboration agreements may place restrictions on our activities with respect to other products, including by limiting our ability to grant licenses or develop products with other third parties, or in different indications, diseases or geographical locations, or may place additional obligations on us with respect to development or commercialization of our product candidates. If we fail to comply with or breach any provision of a collaboration agreement, a collaborator may have the right to terminate, in whole or in part, such agreement or to seek damages.

Some of our collaboration agreements, including the BeiGene Agreement, are complex and involve sharing or division of ownership of certain data, know-how and intellectual property rights among the various parties. Accordingly, our collaborators could interpret certain provisions differently than we or our other collaborators which could lead to unexpected or inadvertent disputes with collaborators. In addition, these agreements might make additional collaborations, partnering or mergers and acquisitions difficult.

There is no assurance that a collaborator who is acquired by a third party would not attempt to change certain contract provisions that could negatively affect our collaboration. The acquiring company may also not accept the terms or assignment of our contracts and may seek to terminate the agreements. Any one of our collaborators could breach covenants, restrictions and/or sub-license agreement provisions leading us into disputes and potential breaches of our agreements with other partners.

If we or third parties are unable to successfully develop companion diagnostics for our product candidates, or experience significant delays in doing so, we may not achieve marketing approval or realize the full commercial potential of such product candidates.

A key part of our development strategy for sitravatinib used as a single agent and mocetinostat is to identify subsets of patients with specific types of tumors that express specific genetic markers. Identification of these patients will require the use and development of companion diagnostics. We expect that the FDA and comparable foreign regulatory authorities will require the regulatory approval of a companion diagnostic as a condition to approving our product candidates for the selection of patients with tumors expressing specific genetic markers. We do not have experience or capabilities in developing or commercializing diagnostics and plan to rely in large part on third parties to perform these functions. We do not currently have any long-term arrangements in place with any third party to develop or commercialize companion diagnostics for our sitravatinib product candidate.

Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as medical devices and will likely require separate regulatory approval prior to commercialization. If we or third parties are unable to successfully develop companion diagnostics for our product candidates, or experience delays in doing so:

- the development of these product candidates may be delayed because it may be difficult to identify patients for enrollment in our clinical trials in a timely manner;

- these product candidates may not receive marketing approval if their safe and effective use depends on a companion diagnostic; and

- we may not realize the full commercial potential of these product candidates that receive marketing approval if, among other reasons, we are unable to appropriately identify patients or types of tumors with the specific genetic alterations targeted by these product candidates.

Even if our product candidates and any associated companion diagnostics are approved for marketing, the need for companion diagnostics may slow or limit adoption of our product candidates. Although we believe genetic testing is becoming more prevalent in the diagnosis and treatment of cancer, our product candidates may be perceived negatively compared to alternative treatments that do not require the use of companion diagnostics, either due to the

additional cost of the companion diagnostic or the need to complete additional procedures to identify genetic markers prior to administering our product candidates.

If any of these events were to occur, our business and growth prospects would be harmed, possibly materially.

We rely upon third-party contractors and service providers for the execution of some aspects of our development programs. Failure of these collaborators to provide services of a suitable quality and within acceptable timeframes may cause the delay or failure of our development programs.

We outsource certain functions, tests and services to CROs, medical institutions and collaborators and outsource manufacturing to collaborators and/or contract manufacturers, and we rely on third parties for quality assurance, clinical monitoring, clinical data management and regulatory expertise. In particular, we rely on CROs to run our clinical trials on our behalf and contract manufacturers to manufacture our product candidates. There is no assurance that such individuals or organizations will be able to provide the functions, tests, drug supply or services as agreed upon or to acceptable quality standards, and we could suffer significant delays in the development of our products or processes.

In some cases, there may be only one or few providers of such services, including clinical data management and manufacturing services. In addition, the cost of such services could increase significantly over time. We rely on third parties as mentioned above to enroll qualified patients and conduct, supervise and monitor our clinical trials. Our reliance on these third parties and collaborators for clinical development activities reduces our control over these activities, but does not relieve us of our regulatory responsibilities, including ensuring that our clinical trials are conducted in accordance with good clinical practices ("GCP") regulations and the investigational plan and protocols contained in the regulatory agency applications. In addition, these third parties may not complete activities on schedule or may not manufacture compounds under GMP conditions. Preclinical studies may not be performed or completed in accordance with good laboratory practices, or GLP, regulatory requirements or our trial design. If we or our CROs fail to comply with GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving any marketing applications. If these third parties or collaborators do not successfully carry out their contractual duties or meet expected deadlines, obtaining regulatory approval for manufacturing and commercialization of our product candidates may be delayed or prevented. We rely substantially on third-party data managers for our clinical trial data. There is no assurance that these third parties will not make errors in the design, management or retention of our data or data systems. There is no assurance that these third parties will pass FDA or regulatory audits, which could delay or prohibit regulatory approval.

Our CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other product development activities, which could harm our competitive position. If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. Further, switching or adding additional CROs involves additional cost and requires management time and attention. In addition, there is a natural transition period when a new CRO commences work. As a result, delays may occur, which could materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

The timelines of our clinical trials may be impacted by numerous factors and any delays may adversely affect our ability to execute our current business strategy.

Clinical testing is expensive, difficult to design and implement, can take many years to complete, and is uncertain as to outcome. We may experience delays in clinical trials at any stage of development and testing of our product candidates. Our planned clinical trials may not begin on time, have an effective design, enroll a sufficient number of subjects, or be completed on schedule, if at all.

Events which may result in a delay or unsuccessful completion of clinical trials include:

- inability to raise funding necessary to initiate or continue a trial;
- delays in obtaining regulatory approval to commence a trial;
- delays in reaching agreement with the FDA on final trial design;
- imposition of a clinical hold following an inspection of our clinical trial operations or trial sites by the FDA or other regulatory authorities;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in obtaining required institutional review board approval at each site;

- delays in having subjects complete participation in a trial or return for post-treatment follow-up;
- delays caused by subjects dropping out of a trial due to side effects or otherwise;
- clinical sites dropping out of a trial to the detriment of enrollment;
- time required to add new clinical sites; and
- delays by our contract manufacturers to produce and deliver a sufficient supply of clinical trial materials.

Furthermore, enrollment may depend on the availability of suitable companion diagnostics to identify genetic markers we are targeting and the capability and willingness of clinical sites to conduct genetic screening of potential patients.

If initiation or completion of any of our clinical trials for our product candidates are delayed for any of the above reasons or for other reasons, our development costs may increase, our approval process could be delayed, any periods after commercial launch and before expiration of patent protection may be reduced and our competitors may have more time to bring products to market before we do. Any of these events could impair the commercial potential of our product candidates and could have a material adverse effect on our business.

If we experience delays or difficulties in the enrollment of patients in clinical trials, those clinical trials could take longer than expected to complete and our receipt of necessary regulatory approvals could be delayed or prevented. We may not be able to initiate or complete clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials. In particular, because we are focused on patients with specific genetic alterations in some of our trials, our pool of suitable patients may be smaller and more selective and our ability to enroll a sufficient number of suitable patients may be limited or take longer than anticipated. For example, our product candidate sitravatinib is a targeted therapeutic candidate to treat patients with cancers that are driven by dysregulated receptor tyrosine kinases ("RTK"), specifically tumors that harbor CBL, CHR4Q12 and RET genetic alterations in non-small cell lung cancer ("NSCLC") and other tumor types. These mutations are estimated to be present in a total of 5.5% of all NSCLC patients. The frequency at which the CBL, CHR4Q12 and RET genetic alterations are expressed in certain tumor types may affect our success in enrolling a suitable number of patients to participate in our clinical trials. In addition, some of our competitors have ongoing clinical trials for product candidates that treat the same indications, including NSCLC, where we are studying each of mocetinostat and sitravatinib in combination with checkpoint inhibitors, or target the same genetic alterations as our product candidates. Therefore, patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment for any of our clinical trials may also be affected by other factors, including without limitation:

- the severity of the disease under investigation
- the frequency of the genetic alteration we are seeking to target in the applicable trial, and the ability to effectively identify such alteration;
- the willingness of clinical sites and principal investigators to subject candidate patients to genetic screening;
- the eligibility criteria for the study in question;
- the perceived risks and benefits of the product candidate under study;
- the availability, effectiveness and safety of other treatment options;



the patient referral practices of physicians;

the ability to monitor patients adequately during and after treatment; and

the proximity and availability of a sufficient number of clinical trial sites that are willing to comply with the requirements of our clinical protocols.

For example, due to the targeted indications and patient populations we intend to focus on for development of our product candidates, the number of study sites and patient populations available to us may be limited, and therefore enrollment of suitable patients to participate in clinical trials for these product candidates may take longer than would be the case if we were pursuing broader indications or patient populations.

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

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Results of our trials could reveal a high and unacceptable severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. Treatment-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial, or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the product label;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

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

We are and continue to be subject to stringent government regulations concerning the clinical testing of our products. We will also continue to be subject to government regulation of any product that receives regulatory approval.

Numerous statutes and regulations govern human testing and the manufacture and sale of human therapeutic products in the United States and other countries where we intend to market our products. Such legislation and regulation bears upon, among other things, the approval of trial protocols and human testing, the approval of manufacturing facilities, testing procedures and controlled research, the review and approval of manufacturing, preclinical and clinical data prior to marketing approval, including adherence to GMP during production and storage, and marketing activities including advertising and labeling.

Clinical trials may be delayed or suspended at any time by us or by the FDA or other similar regulatory authorities if it is determined at any time that patients may be or are being exposed to unacceptable health risks, including the risk of death, or if compounds are not manufactured under acceptable GMP conditions or with acceptable quality. Current regulations relating to regulatory approval may change or become more stringent. The agencies may also require

additional trials be run in order to provide additional information regarding the safety, efficacy or equivalency of any product candidate for which we seek regulatory approval.

Moreover, any regulatory approval of a drug which is eventually obtained may entail limitations on the indicated uses for which that drug may be marketed or on the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA or a comparable foreign regulatory authority approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with GMPs and GCPs for any clinical trials that we conduct post-approval. Furthermore, product approvals may be withdrawn or limited in some way if problems occur following initial marketing or if compliance with regulatory standards is not maintained. Similar restrictions are imposed in foreign markets.

Regulatory agencies could become more risk averse to any side effects or set higher standards of safety and efficacy prior to reviewing or approving a product. This could result in a product not being approved.

If we, or any future marketing collaborators or contract manufacturers, fail to comply with applicable regulatory requirements, we may be subject to sanctions including fines, product recalls or seizures and related publicity requirements, injunctions, total or partial suspension of production, civil penalties, suspension or withdrawals of previously granted regulatory approvals, warning or untitled letters, refusal to approve pending applications for marketing approval of new products or of supplements to approved applications, import or export bans or restrictions, and criminal prosecution and penalties. Any of these penalties could delay or prevent the promotion, marketing or sale of our products and product candidates.

The FDA's policies, and policies of comparable foreign regulatory authorities, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or to adopt new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

We have no experience in clinical or commercial manufacturing and depend on others for the production of our product candidates at suitable levels of quality and quantity. Any problems or delays in the manufacture of our products would have a negative impact on our ability to successfully execute our development and commercialization strategies.

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical drug supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We rely on collaborators and/or third parties for development, scale-up, formulation, optimization, management of clinical trial and commercial scale manufacturing and commercialization. There are no assurances we can scale-up, formulate or manufacture any product candidate in sufficient quantities with acceptable specifications for the conduct of our clinical trials or for the regulatory agencies to grant approval of such product candidate. We have not yet commercialized any products and have no commercial manufacturing experience. To be successful, our products must be properly formulated, scalable, stable and safely manufactured in clinical trial and commercial quantities in compliance with GMP and other regulatory requirements and at acceptable costs. Should any of our suppliers or our collaborators be unable to supply or be delayed in supplying us with sufficient supplies, no assurance can be given that we will be able to find alternative means of supply in a short period of time. Should such parties' operations suffer a material adverse effect, the manufacturing of our products would also be adversely affected. Furthermore, key raw materials could become scarce or unavailable. There may be a limited number of third parties who can manufacture our products. We may not be able to meet specifications previously established for product candidates during scale-up and manufacturing.

Our reliance on third parties to manufacture our product candidates will expose us and our partners to risks including the following, any of which could delay or prevent the commercialization of our products, result in higher costs, or deprive us of potential product revenue:

Contract manufacturers can encounter difficulties in achieving the scale-up, optimization, formulation, or volume production of a compound as well as maintaining quality control with appropriate quality assurance. They may also experience shortages of qualified personnel. Contract manufacturers are required to undergo a satisfactory GMP inspection prior to regulatory approval and are obliged to operate in accordance with FDA, International Council for Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use ("ICH"), European and other nationally mandated GMP regulations and/or guidelines governing manufacturing processes, stability testing, record keeping and quality standards. A failure of these contract manufacturers to follow GMP and to document their

adherence to such practices or failure of an inspection by a regulatory agency may lead to significant delays in the availability of our product candidate materials for clinical study, leading to delays in our trials.

For each of our current product candidates we will initially rely on a limited number of contract manufacturers. Changing these or identifying future manufacturers may be difficult. Changing manufacturers requires re-validation of the manufacturing processes and procedures in accordance with FDA, ICH, European and other mandated GMP regulations and/or guidelines. Such re-validation may be costly and time-consuming. It may be difficult or impossible for us to quickly find replacement manufacturers on acceptable terms, if at all.

Our contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to produce, store and distribute our products successfully.

The successful commercialization of our product candidates, if approved, will depend on achieving market acceptance and we may not be able to gain sufficient acceptance to generate significant revenue.

Even if our product candidates are successfully developed and receive regulatory approval, they may not gain market acceptance among physicians, patients, healthcare payors such as private insurers or governments and other funding parties and the medical community. The degree of market acceptance for any of our products will depend on a number of factors, including:

- demonstration of the clinical efficacy and safety of our products;

- the prevalence and severity of any adverse side effects;

- limitations or warnings contained in the product's approved labeling;

- cost-effectiveness and availability of acceptable pricing;

- competitive product profile versus alternative treatment methods and the superiority of alternative treatment or therapeutics;

- the effectiveness of marketing and distribution methods and support for the products; and

- coverage and reimbursement policies of government and third-party payors to the extent that our products could receive regulatory approval but not be approved for coverage by or receive adequate reimbursement from government and quasi-government agencies or other third-party payors.

Disease indications may be small subsets of a disease that could be parsed into smaller and smaller indications as different subsets of diseases are defined. This increasingly fine characterization of diseases could have negative consequences; including creating an approved indication that is so small as not to have a viable market for us. If future technology allows characterization of a disease in a way that is different from the characterization used for large pivotal studies, it may make those studies invalid or reduce their usefulness, and may require repeating all or a portion of the studies. Future technology may supply better prognostic ability which could reduce the portion of patients projected to need a new therapy. Even after being cleared by regulatory authorities, a product may later be shown to be unsafe or not to have its purported effect, thereby preventing its widespread use or requiring withdrawal from the market.

If we fail to obtain coverage and adequate reimbursement for our products, our revenue-generating ability will be diminished and there is no assurance that the anticipated market for our products will be sustained.

We believe that there will be many different applications for products successfully derived from our technologies and that the anticipated market for products under development will continue to expand. However, due to competition from existing or new products and the yet-to-be established commercial viability of our products, no assurance can be given that these beliefs will prove to be correct. Physicians, patients, formularies, payors or the medical community in general may not accept or utilize any products that we or our collaborative partners may develop. Other drugs may be approved during our clinical testing which could change the accepted treatments for the disease targeted and make our product candidates obsolete.

Our and our collaborators' ability to commercialize our products successfully will depend, in part, on the extent to which coverage and adequate reimbursement for such products and related treatments will be available from

governmental health payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers, managed care plans and other organizations. No assurance can be given that third-party coverage and adequate reimbursement will be available that will allow us to maintain price levels sufficient for the realization of an appropriate return on our investment in product development.

Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and private health insurers, managed care plans and other organizations is critical to new product acceptance. There is no uniform coverage and reimbursement policy among third-party payors in the United States; however, private third-party payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Additionally, coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. Even if we obtain coverage for our product candidates, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In the United States and in many other countries, pricing and/or profitability of some or all prescription pharmaceuticals and biopharmaceuticals are subject to varying degrees of government control. In the United States, there has recently been increased government enforcement and government and payor scrutiny relating to drug pricing and price increases. For example, there have been several recent U.S. Congressional inquiries and proposed and enacted federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump Administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump Administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. These changes may adversely impact the prices we or our future collaborators may charge for our products candidates, if commercialized.

Outside of the United States, the successful commercialization of our products will depend largely on obtaining and maintaining government coverage, because in many countries patients are unlikely to use prescription drugs that are not covered by their government healthcare programs. Negotiating coverage and reimbursement with governmental authorities can delay commercialization by 12 months or more. Coverage and reimbursement policies may adversely affect our ability to sell our products on a profitable basis. In many international markets, governments control the prices of prescription pharmaceuticals, including through the implementation of reference pricing, price cuts, rebates, revenue-related taxes and profit control, and we expect prices of prescription pharmaceuticals to decline over the life of the product or as volumes increase.

Healthcare reform and controls on healthcare spending may limit the price we charge for any products and the amounts thereof that we can sell. In particular, in the United States, the federal government and private insurers have changed and have considered ways to change, the manner in which healthcare services are provided. In March 2010, the ACA became law in the United States. With respect to pharmaceutical products, the ACA, among other things, expanded and increased industry rebates for drugs covered by Medicaid and made changes to the coverage requirements under Medicare Part D, Medicare's prescription drug benefits program. Some of the provisions of the ACA have yet to be fully implemented, and there have been judicial and Congressional challenges to certain aspects of the ACA, as well as recent efforts by the Trump Administration to repeal or replace certain aspects of the ACA. President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additionally, on January 23, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018 (the "BBA") among other things, amends the ACA, effective January 1, 2019, to increase from 50 percent to 70



percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” We cannot predict how the ACA, its possible repeal or replacement or other potential future healthcare reform may impact our operations.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation’s automatic reduction to several government programs. These changes include aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013 and, as amended by subsequent legislation including the BBA, will stay in effect through 2027 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Moreover, the Drug Supply Chain Security Act, enacted in 2013, imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing.

These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

We anticipate that the ACA, as well as alternative or replacement healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and additional downward pressure on the reimbursement we may receive for any approved product. Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives.

In addition, levels of reimbursement may be impacted by other current and future legislation, regulation or reimbursement policies of third-party payors in a manner that may harm the demand and reimbursement available for our products, including for companion diagnostics for our products, which in turn, could harm our future product pricing and sales. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

Competition in our targeted market area is intense and this field is characterized by rapid technological change. Therefore developments by competitors may substantially alter the predicted market or render our product candidates uncompetitive.

There are hundreds of drugs in clinical development today in the area of oncology therapeutics. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. In the oncology market, our major competitors include, but are not limited to: Nektar Therapeutics, Corvus Pharmaceuticals, Inc., Bristol-Myers Squibb, Syndax, Inc., Cabometyx (Exelixis, Inc.) Blueprint Medicines, Inc., Ignyta, Inc., Loxo Oncology, Inc., Acetylon Pharmaceuticals, Inc., Chroma Therapeutics Ltd., Huya Bioscience International, Shenzhen Chipscreen Biosciences Ltd., Celgene Corporation, Curis Inc., MEI Pharma Inc., Merck & Co Inc., Novartis and Pharmacyclics Inc. among others.

Many companies have filed, and continue to file, patent applications in oncology which may or could affect our program. Some of these patent applications may have already been allowed or issued, and others may issue in the future. These companies include, but are not limited to: Bristol-Myers Squibb Company; Compugen Limited; Exelixis; GlaxoSmithKline PLC; Novartis; Pfizer and Araxes Pharma LLC. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed, and additional patents granted, in the future, as well as additional research and development programs expected in the future.

In addition to companies that have HDAC inhibitors or kinase inhibitors addressing oncology indications, our competition also includes hundreds of private and publicly traded companies that operate in the area of oncology but have therapeutics with different mechanisms of action. The oncology market in general is highly competitive with over 1,000 molecules currently in clinical development.

Developments by others may render our products or technologies non-competitive or obsolete or we may not be able to keep pace with technological developments. Our competitors may have developed or may be developing technologies which may be the basis for competitive products. Some of these products may prove to be more effective and less costly than the products developed or being developed by us. Our competitors may obtain regulatory approval for their products more rapidly than we do which may change the standard of care in the indications we are targeting, rendering our technology or products non-competitive or obsolete. For example, with the recent approval of immunotherapy agents for the treatment of NSCLC and other cancers, the standard of care for the treatment of cancer is evolving and will continue to evolve which could require us to change the design and timelines for our registration

trials and may limit the commercial acceptance of our products in the future. Others may develop treatments or cures superior to any therapy we are developing or will develop. Moreover, alternate, less toxic forms of medical treatment may be developed which may be competitive with our products.

Many of the organizations which could be considered to be our competitors have substantially more financial and technical resources, more extensive discovery research, preclinical research and development capabilities and greater manufacturing, marketing, distribution, production and human resources than we do. Many of our current or potential competitors have more experience than us in research, preclinical testing and clinical trials, drug commercialization, manufacturing and marketing, and in obtaining domestic and foreign regulatory approvals. In addition, failure, unacceptable toxicity, lack of sales or disappointing sales or other issues regarding competitors' products or processes could have a material adverse effect on our product candidates, including our clinical candidates or our lead compounds. Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in

efficacy, convenience, tolerability and safety in order to overcome price competition and brand recognition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, receiving FDA, EMA or other regulatory approval or discovering, developing and commercializing medicines before we do, which would have a material adverse impact on our business.

We will not be able to successfully commercialize our product candidates without establishing sales and marketing capabilities internally or through collaborators.

We currently have no sales and marketing staff. We may not be able to find suitable sales and marketing staff and collaborators for all of our product candidates. We have no prior experience in the marketing, sale and distribution of pharmaceutical products and there are significant risks involved in building and managing a sales organization, including our ability to hire, retain and incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any collaborators may not be adequate or successful or could terminate or materially reduce the effort they direct to our products. The development of a marketing and sales capability will require significant expenditures, management resources and time. The cost of establishing such a sales force may exceed any potential product revenue, or our marketing and sales efforts may be unsuccessful. If we are unable to develop an internal marketing and sales capability in a timely fashion, or at all, or if we are unable to enter into a marketing and sales arrangement with a third party on acceptable terms, we may be unable to successfully develop and seek regulatory approval for our product candidates and/or effectively market and sell approved products, if any.

We are subject to competition for our skilled personnel and may experience challenges in identifying and retaining key personnel that could impair our ability to conduct our operations effectively.

Our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified personnel. If we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy. Although we have not experienced problems attracting and retaining highly qualified personnel in the recent past, our industry has experienced a high rate of turnover of management personnel in recent years. Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, especially Charles M. Baum, M.D., Ph.D., our President and Chief Executive Officer, Isan Chen, M.D., our Executive Vice President and Chief Medical and Development Officer, James Christensen, Ph.D. our Chief Scientific Officer, Jamie A. Donadio, our Senior Vice President and Chief Financial Officer, and Chris LeMasters, our Executive Vice President and Chief Business Officer whose services are critical to the successful implementation of our product candidate acquisition, development and regulatory strategies, as well as the management of our financial operations. We are not aware of any present intention of any of these individuals to leave our Company. In order to induce valuable employees to continue their employment with us, we have provided stock options that vest over time. The value to employees of stock options that vest over time is significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies.

Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us at any time, with or without notice. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could harm our business, financial condition and prospects. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level and senior managers as well as junior, mid-level and senior scientific and medical personnel.

We may also experience growth in the number of our employees and the scope of our operations, especially in clinical development. This growth will place a significant strain on our management, operations and financial resources and we may have difficulty managing this future potential growth. No assurance can be provided that we will be able to attract new employees to assist in our growth. Many of the other pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. We also may employ consultants or part-time and contract employees. There can be no assurance that these individuals are retainable. While we have been able to attract and retain skilled and experienced personnel and consultants in the past, no assurance can be given that we will be able to do so in the future.

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

As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Our current and future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal False Claims Act, which may constrain the business or financial arrangements and relationships through which we sell, market and distribute any drugs for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by U.S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. The laws that may affect our ability to operate include:

the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order, lease, furnishing, prescribing or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid. The term "remuneration" has been broadly interpreted to include anything of value. The ACA, among other things, amended the intent requirement of the federal Anti Kickback Statute such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate, in order to commit a violation;

federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Entities can be held liable under these laws if they are deemed to "cause" the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers, promoting a product off label, or for providing medically unnecessary services or items. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act;

the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), which imposes criminal and civil liability for knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of or payment for healthcare benefits, items or services;

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH"), and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in

U.S. federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions;

the federal Open Payments program, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare and Medicaid Services ("CMS") information related to "payments or other transfers of value" made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians and their immediate family members, and contains requirements for manufacturers to submit reports to CMS by the 90th day of each calendar year, and disclosure of such information to be made by CMS on a publicly available website; and

analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exceptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. To the extent that any of our product candidates is ultimately sold in countries other than the United States, we may be subject to similar laws and regulations in those countries. If we or our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil, criminal and administrative penalties, damages, fines, individual imprisonment, disgorgement, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, exclusion from participation in government healthcare programs, and the curtailment or restructuring of our operations, any of which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including any of our collaborators, is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusion from participation in government healthcare programs, which could also materially affect our business.

We may become subject to the risk of product liability claims.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. Human therapeutic products involve the risk of product liability claims and associated adverse publicity. Currently, the principal risks we face relate to patients in our clinical trials, who may suffer unintended consequences. Claims might be made by patients, healthcare providers, pharmaceutical companies or others. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection laws. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates, if approved. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- costs to defend the related litigation;



- diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue from product sales; and
- the inability to commercialize any of our product candidates, if approved.

We may not have or be able to obtain or maintain sufficient and affordable insurance coverage, and without sufficient coverage any claim brought against us could have a materially adverse effect on our business, financial condition or results of

operations. We run clinical trials through investigators that could be negligent through no fault of our own and which could affect patients, cause potential liability claims against us and result in delayed or stopped clinical trials. We are required in many cases by contractual obligations to indemnify collaborators, partners, third-party contractors, clinical investigators and institutions. These indemnifications could result in a material impact due to product liability claims against us and/or these groups. We currently carry \$10 million in product liability insurance, which we believe is appropriate for our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our business involves the controlled use of hazardous materials and as such we are subject to environmental and occupational safety laws. Continued compliance with these laws may incur substantial costs and failure to maintain compliance could result in liability for damages that may exceed our resources.

Our preclinical research, manufacturing and development processes involve the controlled use of hazardous and radioactive materials. We are subject to federal, local and foreign laws and regulations governing the use, manufacture, storage, handling and disposal of such materials and certain waste products. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. The risk of accidental contamination or injury from these materials cannot be completely eliminated. In the event of such an accident, we could be held liable for any damages that result, and any such liability could exceed our resources. We may not be adequately insured against this type of liability. We may be required to incur significant costs to comply with environmental laws and regulations in the future, and our operations, business or assets may be materially adversely affected by current or future environmental laws or regulations.

We may have to dedicate resources to the settlement of litigation.

Securities legislation in the United States, Canada and other countries makes it relatively easy for stockholders to sue. This could lead to frivolous lawsuits which could take substantial time, money, resources and attention or force us to settle such claims rather than seek adequate judicial remedy or dismissal of such claims.

If we are required to defend patent infringement actions brought by third parties, or if we sue to protect our own patent rights or otherwise to protect our proprietary information and to prevent its disclosure, or if we are involved in other litigation, whether as a plaintiff or defendant, we may be required to pay substantial litigation costs and managerial attention may be diverted from business operations even if the outcome is in our favor. If we are required to defend our patents or trademarks against infringement by third parties, we may be required to pay substantial litigation costs and managerial attention and financial resources may be diverted from our research and development operations even if the outcome is in our favor.

We may be vulnerable to disruption, damage and financial obligation as a result of system failures.

Despite the implementation of security measures, any of the internal computer systems belonging to us, our collaborators or our third-party service providers are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failure. Any system failure, accident or security breach that causes interruptions in our own, in collaborators' or in third-party service vendors' operations could result in a material disruption of our drug discovery and development programs. In addition, we rely upon third-party contractors and service providers for the hosting, support and/or maintenance of some aspects of our computer hardware, computer software and telecommunications systems. Failure of those contractors and service providers to

provide systems and services of a suitable quality and within acceptable timeframes may cause the delay or failure of our development programs, or loss of confidential or proprietary information. To the extent that any disruption or security breach results in a loss or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we may incur liability, our drug discovery and development programs may be adversely affected and the further development of our product candidates may be delayed. Furthermore, we may incur additional costs to remedy the damages caused by these disruptions or security breaches.

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

The U.S. government recently enacted comprehensive tax legislation that includes significant changes to the taxation of business entities. These changes include, among others, (i) a permanent reduction to the corporate income tax rate, (ii) a partial limitation on the deductibility of business interest expense, (iii) a shift of the U.S. taxation of multinational corporations from a tax on worldwide income to a territorial system (along with certain rules designed to prevent erosion of the U.S. income tax base) and (iv) a one-time tax on accumulated offshore earnings held in cash and illiquid assets, with the latter taxed at a lower rate.

Notwithstanding the reduction in the corporate income tax rate, the overall impact of this tax reform is uncertain, and our business and financial condition could be adversely affected.

### Risks Relating to Our Intellectual Property

We may not obtain adequate protection for our product candidates through patents and other intellectual property rights and as such our competitive advantage in the marketplace may be compromised.

Our success depends, in part, on our ability to secure and protect our patents, trade secrets, trademarks and other intellectual property rights and to operate without infringing on the proprietary rights of others or having third parties circumvent the rights that we own or license. We have filed and are actively pursuing patent applications in the United States, Japan, Europe and other major markets via the Patent Cooperation Treaty or directly in countries of interest. The patent positions of healthcare companies, universities and biopharmaceutical companies, including ours, are uncertain and involve complex questions of law and fact for which important legal issues may remain unresolved. Therefore, there is no assurance that our pending patent applications will result in the issuance of patents or that we will develop additional proprietary products which are patentable. Moreover, patents issued or to be issued to us may not provide us with any competitive advantage. Further, if the patent applications we hold or in-license with respect to our programs, product candidates and companion diagnostic fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize future products.

Our patents may be challenged by third parties at the United States Patent and Trademark Office ("USPTO"), comparable foreign patent offices, or in patent litigation. In addition, it is possible that third parties with products that are very similar to ours will circumvent our patents by means of alternate designs or processes or file applications or be granted patents that would block or hurt our efforts.

There are no assurances that our patent counsel, lawyers or advisors have given us correct advice or counsel. Opinions from such patent counsel or lawyers may not be correct or may be based on incomplete facts. We cannot be certain that we are the first to invent or first to file for patent protection for the inventions covered by pending patent applications and, if we are not, we may be subject to priority disputes. We may be required to disclaim part or all of the subject matter and/or term of certain patents or all of the subject matter and/or term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of one or more claims, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that if challenged, our patents would be declared by the USPTO, comparable foreign patent offices or a court to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. The possibility exists that others will develop products which have the same effect as our products on an independent basis which do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our products. The steps we have taken to protect our intellectual property may not prevent the misappropriation of our proprietary information and technologies, particularly in foreign countries where laws or law enforcement practices may not protect proprietary rights to the same extent as in the United States, Europe or Japan. Unauthorized disclosure of our proprietary information could also harm our competitive position. We could also inadvertently use our collaborators' data inappropriately which could lead to liability. We may file patent applications but have claims restricted or we may not be able to supply sufficient data to satisfy a patent office to support our claims and, as a result, may not obtain the original claims desired or we may receive restricted claims. Alternatively, it is possible that we may not receive any patent protection from an application.

Maintaining our patents and applications requires timely payment of fees and other associated costs in the countries of filing, and we could inadvertently abandon a patent or patent application (or trademark or trademark application) due to non-payment of fees, or as a result of a failure to comply with filing deadlines or other requirements of the prosecution process, resulting in the loss of protection of certain intellectual property rights in a certain country. Alternatively, we, our collaborators or our patent counsel may take action resulting in a patent or patent application becoming abandoned which may not be able to be reinstated, or if reinstated, may suffer patent term adjustments. Any of these outcomes could hurt our ability to gain full patent protection for our products. Registered trademarks and/or applications for trademark registrations in the United States that belong to us are subject to similar risks as described above for patents and patent applications.

Many of our collaboration agreements, including the BeiGene Agreement, are complex and may call for licensing or cross-licensing of potentially blocking patents, know-how or intellectual property. Due to the potential overlap of data, know-how and intellectual property rights there can be no assurance that one of our collaborators will not dispute our right to send data or know-how or other intellectual property rights to third parties and this may potentially lead to liability or termination of a program or

litigation. There are no assurances that the actions of our collaborators would not lead to disputes or cause us to default with other collaborators. We cannot be certain that a collaborator will not challenge the validity of licensed patents.

We cannot be certain that any country's patent and/or trademark office will not implement new rules which could affect how we draft, file, prosecute and/or maintain patents and patent applications, or that certain patent rights and/or trademark rights will be granted by governmental authorities in particular foreign countries. We cannot be certain that increasing costs for drafting, filing, prosecuting and maintaining patent applications and patents will not limit our ability to file for patent protection, or to prosecute applications through to grant. We may be forced to abandon or return the rights to specific patents due to a lack of financial resources. There is no assurance that we could enter into licensing arrangements at a reasonable cost, or develop or obtain alternative technology in respect of patents issued to third parties that incidentally cover our products. Any inability to secure such licenses or alternative technology could result in delays in the introduction of some of our products or even lead to prohibition of the development, manufacture or sale of certain products by us.

We may file applications for trademark registrations in connection with our product candidates in various jurisdictions, including the United States. No assurance can be given that any of our trademark applications will be registered in the United States or elsewhere, or that the use of any registered or unregistered trademarks will confer a competitive advantage in the marketplace. Furthermore, even if we are successful in our trademark registrations, the FDA and regulatory authorities in other countries have their own process for drug nomenclature and their own views concerning appropriate proprietary names. No assurance can be given that the FDA or any other comparable regulatory authority will accept any of our trademarks or will not request reconsideration of one of our trademarks, for use in connection with our drug product candidates, whether currently or at some time in the future. The loss, abandonment, or cancellation of any of our trademarks or trademark applications could negatively affect the success of the product candidates to which they relate.

Moreover, some of our know-how and technology which is not patented or not patentable may constitute trade secrets. Therefore, we require our consultants, advisors and collaborators to enter into confidentiality agreements and our employees to enter into invention and non-disclosure agreements. However, no assurance can be given that such agreements will provide for a meaningful protection of our trade secrets, know-how or other proprietary information in the event of any unauthorized use or disclosure of information. Furthermore, we cannot provide assurance that any of our employees, consultants, contract personnel or collaborators, either accidentally or through willful misconduct, will not cause serious negative impact to our programs and/or our strategy. All of our employees have signed confidentiality agreements, but there can be no assurance that they will not inadvertently or through their misconduct give trade secrets away.

Third-party patents or intellectual property infringement claims may result in a reduction in the scope of our patent protection and competitive exclusivity with respect to our product candidates. Patent litigation, including defense against third-party intellectual property claims, may result in us incurring substantial costs.

Patent applications which may relate to or affect our business may have been filed by others. Such patent applications or patents resulting there from may conflict with our technologies, patents or patent applications, potentially reducing the scope or strength of our patent protection, and may ultimately be determined to limit or prohibit our freedom to operate with respect to our product candidates. Such events could cause us to stop or change the course of our research and development or modify our intellectual property strategies. We could also become involved in interference proceedings in connection with one or more of our patents or patent applications to determine priority of invention, or in post-grant opposition proceedings at the USPTO or comparable foreign patent offices. There can be no guarantees that an interference proceeding or defense of a post-grant opposition would be successful or that such an outcome would be upheld on appeal. An unfavorable outcome could require us to cease using the related technology or to

attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of such interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees.

No assurance can be given that our patents, once issued, would be declared by a court to be valid or enforceable, or that we would not be found to infringe a competitor's patent.

Third parties may assert that we are using their proprietary information without authorization. Third parties may also have or obtain patents and may claim that technologies licensed to or used by us infringe their patents. Because patent applications can take many years to issue, third parties may have currently pending patent applications which may later result in issued patents that our product candidates or companion diagnostic may infringe, or which such third parties claim are infringed by the use of our technologies. If any third-party patents are held by a court of competent jurisdiction to cover any aspect of our product candidates, including the formulation or method of use of such product candidate, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtain a license under the applicable patents, or until such patents expire. In any such case, such a license may not be available on commercially reasonable terms or at all. We may attempt to

invalidate a competitor's patent or trademark. There is no assurance such action will ultimately be successful and, even if initially successful, it could be overturned upon appeal. In addition, any legal action that seeks damages or an injunction to stop us from carrying on our commercial activities relating to the affected technologies could subject us to monetary liability. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources.

Parties making claims against us for alleged infringement of their intellectual property rights may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we could be required to redesign our infringing products or obtain a license from such third party to continue developing and commercializing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. It may be impossible to redesign our products and technology, or it may require substantial time and expense, which could force us to cease commercialization of one or more of our product candidates, or some of our business operations, which could materially harm our business. In addition, in any such proceeding, we may be required to pay substantial damages, including treble damages and attorneys' fees in the event we are found liable for willful infringement.

Our intellectual property may be infringed upon by a third party.

Third parties may infringe one or more of our issued patents or trademarks. We cannot predict if, when or where a third party may infringe one or more of our issued patents or trademarks. There is no assurance that we would be successful in a court of law to prove that a third party is infringing one or more of our issued patents. Even if we are successful in proving in a court of law that a third party is infringing one or more of our issued patents there can be no assurance that we would be successful in halting their infringing activities, for example, through a permanent injunction, or that we would be fully or even partially financially compensated for any harm to our business. We may be forced to enter into a license or other agreement with the infringing third party at terms less profitable or otherwise less commercially acceptable to us than if the license or agreement were negotiated under conditions between those of a willing licensee and a willing licensor. We may not become aware of a third party infringer within legal timeframes that would enable us to seek adequate compensation, or at all, thereby possibly losing the ability to be compensated for any harm to our business. Such a third-party may be operating in a foreign country where the infringer is difficult to locate, where we do not have issued patents and/or the patent laws may be more difficult to enforce. Some third-party infringers may be able to sustain the costs of complex patent infringement litigation more effectively than we can because they have substantially greater resources. Any inability to stop third-party infringement could result in loss in market share of some of our products or even lead to a delay, reduction and/or inhibition of the development, manufacture or sale of certain products by us. There is no assurance that a product produced and sold by a third-party infringer would meet our or other regulatory standards or would be safe for use. Such third-party infringer products could irreparably harm the reputation of our products thereby resulting in substantial loss in market share and profits.

Third parties may seek to obtain approval of a generic version of approved products. Defense against entry of a generic product may result in us incurring substantial costs and ultimate failure to prevail against approval of a generic product could result in a substantial loss of market share and profits.

Even if we are successful in obtaining regulatory approval to sell any of our product candidates in one or more countries, we cannot be certain that our patents and other intellectual property rights will ultimately prevent approval during the patent term of generic products developed and commercialized by third parties. A generic manufacturer may seek approval of a generic version of any of our products in the United States by filing an Abbreviated New Drug Application



("ANDA"), with the FDA asserting that our patents are invalid and/or unenforceable to maintain market exclusivity for any of our products, if approved. We cannot predict if, or when, one or more generic manufacturers may attempt to seek regulatory approval for a generic version of any of our products, if approved. There is no assurance that we will ultimately be successful in a court of law to prevent entry of a generic version of any of our products during the applicable patent term and we may incur substantial costs defending our patents and intellectual property rights. An inability to stop a generic manufacturer from selling a generic version of our products could result in a substantial loss of market share and profits or even preclude the ability to continue to commercialize any of our products, if approved.

## Risks Related to Our Shares of Common Stock

Our share price is volatile and may be influenced by numerous factors that are beyond our control.

A low share price and low market valuation may make it difficult to raise sufficient additional cash due to the significant dilution to current stockholders. Market prices for shares of biotechnology and biopharmaceutical companies such as ours are often volatile. Factors such as clinical and regulatory developments regarding our products or processes, developments regarding potential or future third-party collaborators, announcements of technological innovations, new commercial products, patents, the development of proprietary rights by us or by others or any litigation relating to these rights, regulatory actions, general conditions in the biotechnology and pharmaceutical industries, failure to meet analysts' expectations, publications, financial results or public concern over the safety of biopharmaceutical and biotechnological products, economic conditions in the United States and other countries, terrorism and other factors could have a significant effect on the share price for our shares of common stock. Any setback or delay in the clinical development of our programs could result in a significant decrease in our share price. In recent years the stock of other biotechnology and biopharmaceutical companies has experienced extreme price fluctuations that have been unrelated to the operating performance of the affected companies. There can be no assurance that the market price of our shares of common stock will not experience significant fluctuations in the future, including fluctuations that are unrelated to our performance. These fluctuations may result due to macroeconomic and world events, national or local events, general perception of the biotechnology industry or to a lack of liquidity. In addition, other biotechnology companies' or our competitors' programs could have positive or negative results that impact their stock prices and their results or experience stock price fluctuations that could have a positive or negative impact on our stock price, regardless whether such impact is direct or not.

Stockholders may not agree with our business, scientific, clinical and financial strategy, including additional dilutive financings, and may decide to sell their shares or vote against such proposals. Such actions could materially impact our stock price. In addition, portfolio managers of funds or large investors can change or change their view on us and decide to sell our shares. These actions could have a material impact on our stock price. In order to complete a financing, or for other business reasons, we may elect to consolidate our shares of common stock. Investors may not agree with these actions and may sell our shares. We may have little or no ability to impact or alter such decisions.

Our principal stockholders control the majority of our shares, and their actions may significantly influence matters submitted to our stockholders for approval and our share price.

Based on the information available to us as of December 31, 2017, our stockholders and their affiliates who owned more than 5% of our outstanding common stock collectively owned 53% of our outstanding common stock. Baker Bros. Advisors, L.L.C. ("Baker Brothers") and Boxer Capital, LLC ("Boxer Capital") and their affiliates collectively own 28% of our outstanding common stock. In addition, in conjunction with certain financing transactions, we granted to Baker Brothers and Boxer Capital each the right to nominate a member of our Board of Directors and the right to appoint an observer on our Board of Directors. Collectively Baker Brothers and Boxer Capital may have significant influence over matters submitted to our stockholders for approval, including the election and removal of directors and the approval of any merger, consolidation, or sale of all or substantially all of our assets. Furthermore, as a thinly traded stock, if Baker Brothers, Boxer Capital or any other of our major stockholders determine to exit from the industry or from their holdings in us, for whatever reason, the impact on our share price could be detrimental over a prolonged period of time.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2013 Equity Incentive Plan ("the 2013 Plan"), and our 2013 Employee Stock Purchase Plan ("the ESPP"), our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants, and to sell our common stock to our employees, respectively. Any increase in the number of shares outstanding as a result of the exercise of outstanding options, the vesting or settlement of outstanding stock awards, or the purchase of shares pursuant to the ESPP will cause our stockholders to experience additional dilution, which could cause our stock price to fall.

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

Under Section 382 of the Internal Revenue Code of 1986, as amended ("the Code"), if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three year period, the corporation's ability to use its pre-change U.S. net operating loss carryforwards ("NOLs"), and other pre-change U.S. tax attributes (such as research tax credits) to offset its post-change income may be limited. We experienced an ownership change based on past financing transactions and may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change U.S. net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, would be our stockholders' only source of gain.

We have never declared or paid any cash dividends on our common shares, and we currently expect that earnings, if any, and cash flow will primarily be retained and used in our operations, including servicing any debt obligations we may have now or in the future. Accordingly, although we do not anticipate paying any dividends in the foreseeable future, we may not be able to generate sufficient cash flow in order to allow us to pay future dividends on, or make any distributions with respect to our common stock. As a result, capital appreciation, if any, of our common stock would be our stockholders' sole source of gain on their investment in our common stock for the foreseeable future.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our corporate headquarters is located at 9393 Towne Centre Drive, San Diego, California 92121 where we occupy approximately 18,000 square feet of office and lab space. The lease will expire on January 31, 2019. We believe that our existing facilities are adequate to meet our current needs.

Item 3. Legal Proceedings

None.

Item 4. Mine Safety Disclosures

Not applicable.

## PART II

## Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Our common stock has been listed on The NASDAQ Capital Market since July 15, 2013 under the symbol "MRTX". Prior to that date, there was no public market for our common stock in the United States as our common stock was listed on the Toronto Stock Exchange.

On March 5, 2018, the last reported sale price for our common stock on The NASDAQ Capital Market was \$31.10 per share. The following table sets forth the range of high and low sales prices per share of our common stock as reported on The NASDAQ Capital Market for the period indicated.

|                              | High    | Low     |
|------------------------------|---------|---------|
| Year Ended December 31, 2017 |         |         |
| Fourth Quarter               | \$19.70 | \$11.50 |
| Third Quarter                | \$12.40 | \$3.10  |
| Second Quarter               | \$5.25  | \$2.70  |
| First Quarter                | \$6.30  | \$4.75  |
| Year Ended December 31, 2016 |         |         |
| Fourth Quarter               | \$6.70  | \$4.60  |
| Third Quarter                | \$7.22  | \$4.40  |
| Second Quarter               | \$24.43 | \$5.31  |
| First Quarter                | \$30.85 | \$17.94 |

As of March 5, 2018, we had 18 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers. The actual number of common stockholders is greater than the number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities. We have never declared or paid any cash dividends on our capital stock. We currently intend to retain any future earnings for funding operations and, therefore, do not anticipate paying any cash dividends in the foreseeable future.

#### Stock Performance Graph and Cumulative Total Return

The graph below shows the cumulative total stockholder return assuming the investment of \$100 on July 15, 2013 (and the reinvestment of dividends thereafter) in each of (i) Mirati Therapeutic, Inc.'s common stock, (ii) the NASDAQ Composite Index and (iii) the NASDAQ Biotechnology Index. The comparisons in the graph below are based upon historical data and are not indicative of, or intended to forecast, future performance of our common stock or Indexes.

#### Recent Sales of Unregistered Securities

During the twelve months ended December 31, 2017, we issued and sold the following unregistered securities:

##### Warrant exercise

In 2012, we issued common stock warrants in connection with the issuance of common stock through a private placement. The warrant certificates provide that the warrant holder may elect to exercise their warrant and, in lieu of making the cash payment upon such exercise, receive upon such exercise a net number of common shares determined according to a formula prescribed in the warrant certificate. For the twelve months ended December 31, 2017, all of the remaining outstanding warrants issued in 2012 were exercised, resulting in the issuance of an aggregate of 638,554 shares of our common stock. Three holders of warrants to purchase an aggregate of 109,654 shares of common stock elected to net exercise their warrants, resulting in the issuance of 52,825 shares of our common stock, and six holders of warrants elected to exercise their warrants for cash, generating proceeds of \$4.6 million, and resulting in the issuance of 585,729 shares of our common stock.

The issuances of the securities described above were deemed to be exempt from registration under the Securities Act of 1933, as amended, in reliance on Rule 506 of Regulation D in that each issuance of securities was to an accredited investor under Rule 501 of Regulation D and did not involve a public offering. The recipients of securities in each of these transactions acquired the securities for investment only and not with a view to or for sale in connection with any distribution thereof and appropriate legends were affixed to the securities issued in these transactions. There were no underwriters employed in connection with any of the transactions set forth above.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

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## Item 6. Selected Consolidated Financial Data

The following table presents selected historical financial data for the years ended December 31, 2017, 2016, 2015, 2014 and 2013. All the selected historical financial data has been derived from our Audited Consolidated Financial Statements and is stated in thousands except for per share information.

Please read the following selected financial data in conjunction with Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our Audited Consolidated Financial Statements and related Notes thereto included elsewhere in this Annual Report on Form 10-K.

|                                                               | Year Ended December 31, |            |            |            |            |
|---------------------------------------------------------------|-------------------------|------------|------------|------------|------------|
|                                                               | 2017                    | 2016       | 2015       | 2014       | 2013       |
| <b>Statements of Operations Data:</b>                         |                         |            |            |            |            |
| Loss from operations                                          | \$(71,535)              | \$(83,779) | \$(64,714) | \$(39,104) | \$(31,999) |
| Net loss                                                      | (70,430)                | (83,118)   | (64,544)   | (43,698)   | (52,859)   |
| Comprehensive loss                                            | (70,484)                | (83,143)   | (64,507)   | (43,684)   | (52,872)   |
| Basic and diluted net loss per share                          | \$(2.78)                | \$(4.20)   | \$(3.82)   | \$(3.24)   | \$(4.78)   |
| Weighted average common shares outstanding, basic and diluted | 25,290,222              | 19,787,349 | 16,901,826 | 13,483,467 | 11,057,040 |

|                                                   | As of December 31, |           |           |           |           |
|---------------------------------------------------|--------------------|-----------|-----------|-----------|-----------|
|                                                   | 2017               | 2016      | 2015      | 2014      | 2013      |
| <b>Balance Sheet Data:</b>                        |                    |           |           |           |           |
| Cash, cash equivalents and short-term investments | \$150,837          | \$56,734  | \$122,327 | \$29,303  | \$62,070  |
| Working capital                                   | 142,115            | 44,553    | 115,604   | 27,261    | 25,563    |
| Total assets                                      | 157,246            | 63,444    | 128,017   | 33,479    | 64,537    |
| Accumulated deficit                               | (460,627)          | (389,751) | (306,633) | (242,089) | (198,391) |
| Total stockholders' equity                        | 143,288            | 48,309    | 118,176   | 28,062    | 25,885    |

## Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

References in the following discussion to "we", "our", "us", "Mirati" or "the Company" refer to Mirati Therapeutics, Inc. and its subsidiaries.

### Company Overview

Mirati Therapeutics, Inc. is a clinical-stage oncology company developing targeted therapeutics to address the genetic, epigenetic and immunological promoters of cancer. We were incorporated under the laws of the State of Delaware on April 29, 2013 as Mirati Therapeutics, Inc. and our corporate headquarters is located in San Diego, California. Our precision oncology clinical programs utilize next-generation genomic testing to identify and select cancer patients who are most likely to benefit from targeted drug treatment. In immuno-oncology, we are advancing clinical programs where the ability of our product candidates to improve the immune environment of tumor cells may enhance and expand the efficacy of existing cancer immunotherapy medicines when given in combination. Our preclinical programs include product candidates specifically designed to address mutations and tumors where few treatment options exist. We approach each of our discovery and development programs with a singular focus: to translate our deep understanding of the molecular drivers of cancer into better therapies and better outcomes for patients.

Our clinical pipeline consists of two clinical-stage product candidates: sitravatinib and mocetinostat. We also have a KRAS G12C inhibitor program in preclinical development. The current status and expected 2018 milestones for each program is described below.

#### Sitravatinib

Sitravatinib is being evaluated in multiple clinical trials as an immunotherapy in combination with an immune checkpoint inhibitor in NSCLC and as a targeted agent in patients with specific genetic mutations in NSCLC and other solid tumors.

#### Program Updates

##### Sitravatinib in Combination with Immune Checkpoint Inhibitors

We reported data from the ongoing clinical trial combining sitravatinib and nivolumab at the IASLC 18th World Conference on Lung Cancer, including that three of 11 evaluable patients who had relapsed after previous checkpoint inhibitor treatment experienced a confirmed partial response and seven of the 11 evaluable patients were continuing to receive treatment in the clinical trial, with treatment duration ranging from four months to 10.5 months. We expect to report further data on checkpoint inhibitor refractory patients from this clinical trial in mid-2018.

##### Sitravatinib as a Targeted Agent

We reported early data from an ongoing Phase 1b expansion clinical trial in January 2017 showing that as of December 9, 2016, of the four evaluable patients with RET genetic alterations at the time, there was one patient with stable disease, one unconfirmed partial response and one confirmed partial response. In September 2017, we presented a case study at the IASLC 2017 Chicago Multidisciplinary Symposium in Thoracic Oncology of an NSCLC patient with a CBL inactivating mutation. The case was the first evaluable NSCLC patient harboring a CBL mutation treated in the ongoing Phase 1b study of sitravatinib as a single agent and represents the first example of clinical activity for sitravatinib in a patient with a CBL mutation. We expect to provide an update on this clinical trial in mid-2018.

Collaboration with BeiGene, Ltd. to Develop and Commercialize Sitravatinib in Certain Asian Territories

In January 2018, we entered into a Collaboration and License Agreement (the “BeiGene Agreement”) with BeiGene, Ltd. (“BeiGene”), pursuant to which Mirati and BeiGene agreed to collaboratively develop sitravatinib in Asia (excluding Japan and certain other countries), Australia and New Zealand (the “Licensed Territory”). Under the BeiGene Agreement, Mirati granted BeiGene an exclusive license to develop, manufacture and commercialize sitravatinib in the Licensed Territory, with Mirati retaining exclusive rights for the development, manufacturing and commercialization of sitravatinib outside the Licensed Territory.

Under the BeiGene Agreement, BeiGene agreed to pay to Mirati an upfront fee of \$10.0 million. BeiGene is also required to make milestone payments to Mirati of up to an aggregate of \$123.0 million upon the first achievement of specified clinical, regulatory and sales milestones. Additionally, BeiGene is obligated to pay to Mirati royalties at tiered percentage rates ranging from mid-single digits to twenty percent on annual net sales of licensed products in the Licensed Territory, subject to reduction under specified circumstances.

#### Mocetinostat

Mocetinostat is being evaluated in a Phase 2 clinical trial in combination with durvalumab (IMFINZI™), MedImmune Limited's anti-PD-L1 inhibitor, for the treatment of patients with NSCLC. Stage 1 of the trial is currently enrolling nine patients in each cohort; one cohort has already met the prespecified criteria for expansion into stage 2 with at least one confirmed partial response. We expect to provide an update on stage 1 of this trial in mid-2018.

#### KRAS G12C Inhibitor Program

In November 2017, we announced that Investigational New Drug ("IND") enabling preclinical studies were underway, and an IND submission for the KRAS G12C program is expected by the fourth quarter of 2018, with early clinical proof-of-concept data anticipated in 2019.

#### Glesatinib

In November 2017, we announced that, in light of superior investment opportunities in our pipeline, we suspended further investment in glesatinib and will pursue opportunities to partner the program.

#### Liquidity Overview

At December 31, 2017, we had \$150.8 million of cash, cash equivalents and short-term investments compared to \$56.7 million at December 31, 2016. In January 2017 and November 2017, we completed public offerings of our common stock and pre-funded common stock warrants that generated net proceeds of \$66.8 million and \$86.7 million, respectively. In January 2018 we received an upfront fee of \$10.0 million in connection with the BeiGene Agreement. We have not generated any revenue from product sales. To date, we have funded our operations primarily through the sale of our common stock and through up-front payments, research funding and milestone payments under collaborative arrangements. To fund future operations, we will likely need to raise additional capital as discussed more fully below under the heading "Liquidity and Capital Resources."

#### Critical Accounting Policies and Significant Judgments and Estimates

Our discussion and analysis of financial condition and results of operations are based upon our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make significant estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and related disclosures. On an ongoing basis, our actual results may differ significantly from our estimates.

While our significant accounting policies are more fully described in Note 2 to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies to be critical to the judgments and estimates used in the preparation of our consolidated financial statements.

#### Accrued Research and Development Expenses

We accrue and expense clinical trial activities performed by third parties based upon estimates of the proportion of work completed over the life of the individual clinical trial and patient enrollment rates in accordance with agreements established with Clinical Research Organizations ("CROs") and clinical trial sites. We determine the estimates by reviewing contracts, vendor agreements and purchase orders, and through discussions with internal clinical personnel and external service providers as to the progress or stage of completion of trials or services and the agreed-upon fee to be paid for such services. However, actual costs and timing of clinical trials are highly uncertain, subject to risks and may change depending upon a number of factors, including our clinical development plan.

We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. If the actual timing of the performance of services or the level of effort varies from the estimate, we will adjust the accrual accordingly. Nonrefundable advance payments for goods and services, including fees for process development or manufacturing and distribution of clinical supplies that will be used in future research and development activities, are deferred and recognized as expense in the period that the related goods are consumed or services are performed.

## Share-Based Compensation

We measure and recognize compensation expense for share-based payments based on estimated fair value. We estimate the fair value of stock options granted using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of certain estimates and judgmental assumptions that affect the amount of share-based compensation expense recognized in our consolidated financial statements. These assumptions include the historical volatility of our stock price, expected term of the options, the risk-free interest rate and expected dividend yields. Share-based compensation is recognized using the graded accelerated vesting method. If any of the assumptions used in our calculation change significantly, share-based compensation expense may differ materially from what we have recorded in the current period.

## Financial Operations Overview

### Research and Development Expenses

Research and development expenses consist primarily of:

- salaries and related expenses for personnel, including expenses related to stock options or other share-based compensation granted to personnel in development functions;
- fees paid to external service providers such as CROs and contract manufacturing organizations related to clinical trials, including contractual obligations for clinical development, clinical sites, manufacturing and scale-up, and formulation of clinical drug supplies; and
- costs for allocated facilities and depreciation of equipment.

We record research and development expenses as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expense when the services have been performed or when the goods have been received. At this time, due to the risks inherent in the clinical development process and the early stage of our product development programs we are unable to estimate with any certainty the costs we will incur in the continued development of sitravatinib and mocetinostat. The process of conducting clinical trials necessary to obtain regulatory approval and manufacturing scale-up to support expanded development and potential future commercialization is costly and time consuming. Any failure by us or delay in completing clinical trials, manufacturing scale up or in obtaining regulatory approvals could lead to increased research and development expense and, in turn, have a material adverse effect on our results of operations. We expect that our research and development expenses may increase if we are successful in advancing sitravatinib, mocetinostat, our preclinical KRAS G12C program, or any of our other preclinical programs into more advanced stages of clinical development.

### General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related benefits, including share-based compensation, related to our executive, finance, business development, legal and support functions. Other general and administrative expenses include professional fees for auditing and tax services, rent and utilities and insurance.

## Results of Operations

### Comparison of the Years Ended December 31, 2017 and 2016

The following table summarizes our results of operations for the year ended December 31, 2017 and 2016 (in thousands):

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|                                     | Year Ended<br>December 31, |          | Increase    |
|-------------------------------------|----------------------------|----------|-------------|
|                                     | 2017                       | 2016     | (Decrease)  |
| Research and development expenses   | \$58,085                   | \$68,487 | \$(10,402 ) |
| General and administrative expenses | 13,450                     | 15,292   | (1,842 )    |
| Other income, net                   | 1,105                      | 661      | 444         |

## Research and Development Expenses

Our research and development efforts during the years ended December 31, 2017 and 2016 were focused primarily on our oncology programs, including glesatinib, sitravatinib, mocetinostat, and our preclinical KRAS inhibitor program. The following table summarizes our research and development expenses, (in thousands):

|                                                     | Year Ended<br>December 31, |          | Increase<br>(Decrease) |
|-----------------------------------------------------|----------------------------|----------|------------------------|
|                                                     | 2017                       | 2016     |                        |
| Third-party research and development expenses:      |                            |          |                        |
| Glesatinib                                          | \$17,177                   | \$29,974 | \$(12,797 )            |
| Sitravatinib                                        | 12,282                     | 7,346    | 4,936                  |
| Mocetinostat                                        | 4,883                      | 4,613    | 270                    |
| Preclinical and early discovery                     | 9,148                      | 9,492    | (344 )                 |
| Total third-party research and development expenses | 43,490                     | 51,425   | (7,935 )               |
| Salaries and other employee related expense         | 9,007                      | 8,963    | 44                     |
| Share-based compensation expense                    | 3,192                      | 5,461    | (2,269 )               |
| Other research & development costs                  | 2,396                      | 2,638    | (242 )                 |
| Research and development expense                    | \$58,085                   | \$68,487 | \$(10,402 )            |

Research and development expenses for the year ended December 31, 2017 were \$58.1 million compared to \$68.5 million during the year ended December 31, 2016. The decrease of \$10.4 million during the year ended December 31, 2017 relates to a decrease in third-party research and development expense of \$7.9 million and a decrease in share-based compensation expense. The decrease in third-party research and development expense relates to a decrease in expenses associated with development for glesatinib of \$12.8 million primarily related to decreased manufacturing costs, partially offset by a \$4.9 million increase in sitravatinib development expenses due to the continuation and expansion of ongoing clinical trials. The decrease in share-based compensation expense of \$2.3 million is due to a decrease in the fair value of stock options granted during the year ended December 31, 2017 compared to the year ended December 31, 2016.

## General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2017 were \$13.5 million compared to \$15.3 million for the same period in 2016. The decrease is primarily due to a decrease in share-based compensation expense, which is due to a lower fair value of stock options granted during the year ended December 31, 2017 compared to the year ended December 31, 2016.

## Other Income, Net

Other income, net consisted primarily of interest income of \$1.1 million for the year ended December 31, 2017 and \$0.7 million for the year ended December 31, 2016.

## Comparison of the Years Ended December 31, 2016 and 2015

The following table summarizes the results of our operations for the years ended December 31, 2016 and 2015 (in thousands):

|                               | Year Ended<br>December 31, |          | Increase<br>(Decrease) |
|-------------------------------|----------------------------|----------|------------------------|
|                               | 2016                       | 2015     |                        |
| Research and development, net | \$68,487                   | \$48,959 | \$ 19,528              |



|                             |        |        |      |   |
|-----------------------------|--------|--------|------|---|
| General and administrative  | 15,292 | 15,755 | (463 | ) |
| Other income (expense), net | 661    | 170    | 491  |   |

## Research and Development Expenses

Our research and development efforts during the years ended December 31, 2016 and 2015 were focused primarily on our oncology programs, including glesatinib, sitravatinib, mocetinostat and our preclinical KRAS inhibitor program. The following table summarizes our research and development expenses, (in thousands):

|                                                     | Year Ended<br>December 31, |          | Increase<br>(Decrease) |
|-----------------------------------------------------|----------------------------|----------|------------------------|
|                                                     | 2016                       | 2015     |                        |
| Third-party research and development expenses:      |                            |          |                        |
| Glesatinib                                          | \$29,974                   | \$21,699 | \$8,275                |
| Sitravatinib                                        | 7,346                      | 3,250    | 4,096                  |
| Mocetinostat                                        | 4,613                      | 5,371    | (758 )                 |
| Preclinical and early discovery                     | 9,492                      | 6,830    | 2,662                  |
| Total third-party research and development expenses | 51,425                     | 37,150   | 14,275                 |
| Salaries and other employee related expense         | 8,963                      | 6,579    | 2,384                  |
| Share-based compensation expense                    | 5,461                      | 3,669    | 1,792                  |
| Other research & development costs                  | 2,638                      | 1,561    | 1,077                  |
| Research and development expense                    | \$68,487                   | \$48,959 | \$19,528               |

Research and development expenses for the year ended December 31, 2016 were \$68.5 million compared to \$49.0 million during the year ended December 31, 2015. The increase of \$19.5 million during the year ended December 31, 2016 primarily relates to an increase in third-party research and development expense of \$14.3 million. The increase in third-party research and development expense relates to increased expenses for glesatinib of \$8.3 million due to costs associated with a Phase 2 clinical trial which began in late 2015, increased expenses for sitravatinib of \$4.1 million due to costs associated with an ongoing Phase 1b clinical trial and increased expenses for our preclinical and early discovery expenses of \$2.7 million due to a one-time license fee of \$2.5 million

The increase in salaries and related expense of \$2.4 million and share-based compensation of \$1.8 million resulted from an increase in the number of research and development employees during the twelve months ended December 31, 2016 compared to the same period of 2015.

## General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2016 were \$15.3 million compared to \$15.8 million for the same period in 2015. The comparable level of expenses for the years ended December 31, 2016 and 2015 reflect a consistent level of general and administrative activities in both years.

## Other Income, Net

Other income, net consisted primarily of interest income of \$0.7 million for the year ended December 31, 2016 and \$0.2 million for the year ended December 31, 2015.

## Liquidity and Capital Resources

To date, we have funded our operations primarily through the sale of our common stock, pre-funded warrants to purchase our common stock, and to a lesser extent through up-front payments, research funding and milestone payments under collaborative arrangements. Since inception, we have primarily devoted our resources to funding research and development programs, including discovery research, preclinical and clinical development activities.

At December 31, 2017, we had \$150.8 million of cash, cash equivalents and short-term investments compared to \$56.7 million at December 31, 2016. In January 2017 and November 2017 we completed public offerings of our common stock and pre-funded common stock warrants that generated net proceeds of \$66.8 million and \$86.7 million, respectively.

We have incurred losses in each year since our inception. Our net losses were \$70.4 million, \$83.1 million and \$64.5 million for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, we had an accumulated

deficit of \$460.6 million. Substantially all of our operating losses resulted from expenses incurred in connection with our product development programs, our research activities and general and administrative costs associated with our operations.

Based on our current and anticipated level of operations, we believe that our cash, cash equivalents and short-term investments will be sufficient to meet our anticipated obligations for at least one year from the date this Annual Report on Form 10-K is filed with the SEC. To fund future operations, we will likely need to raise additional capital. The amount and timing of future funding requirements will depend on many factors, including the timing and results of our ongoing development efforts, the potential expansion of our current development programs, potential new development programs and related general and administrative support. We anticipate that we will seek to fund our operations through public or private equity or debt financings or other sources, such as potential collaboration agreements. We cannot make assurances that anticipated additional financing will be available to us on favorable terms, or at all. Although we have previously been successful in obtaining financing through our equity securities offerings, there can be no assurance that we will be able to do so in the future.

The following table provides a summary of the net cash flow activity for each of the periods set forth below (in thousands):

|                                                     | Year Ended December 31, |            |            |
|-----------------------------------------------------|-------------------------|------------|------------|
|                                                     | 2017                    | 2016       | 2015       |
| Net cash used in operating activities               | \$(64,706)              | \$(68,017) | \$(50,714) |
| Net cash provided by (used in) investing activities | (8,651 )                | 38,255     | (50,753 )  |
| Net cash provided by financing activities           | 158,677                 | 2,652      | 144,367    |
| Increase (decrease) in cash                         | 85,320                  | (27,110 )  | 42,900     |

#### Net cash used in operating activities

Net cash used for operating activities was \$64.7 million, \$68.0 million and \$50.7 million for the years ended December 31, 2017, 2016 and 2015, respectively. Cash used in operating activities during 2017 primarily related to our net loss of \$70.4 million, adjusted for non-cash share-based compensation expense of \$6.8 million and net cash outflows from a change in our operating assets and liabilities of \$1.0 million. Cash used in operating activities during 2016 primarily related to our net loss of \$83.1 million, adjusted for non-cash share-based compensation expense of \$10.6 million and net cash inflows from a change in our operating assets and liabilities of \$4.3 million. Cash used in operating activities during 2015 primarily related to our net loss of \$64.5 million, adjusted for non-cash items such as share-based compensation expense of \$10.3 million and amortization of premium on investments of \$0.3 million, and net cash inflows from a change in our operating assets and liabilities of \$3.0 million.

#### Net cash provided by (used in) investing activities

Investing activities used cash of \$8.7 million for the year ended December 31, 2017, provided cash of \$38.3 million for the year ended December 31, 2016, and used cash of \$50.8 million for the year ended December 31, 2015. The net cash provided or used by investing activities during the periods presented primarily reflects the purchases, sales and maturities of short-term investments.

#### Net cash provided by financing activities

Net cash provided by financing activities was \$158.7 million, \$2.7 million and \$144.4 million for the years ended December 31, 2017, 2016 and 2015, respectively. Net cash provided by financing activities during 2017 consists of net proceeds from the issuance of common stock from our 2017 public offerings of common stock and pre-funded warrants totaling \$153.5 million, proceeds from the exercise of stock options and warrants of \$5.0 million and

proceeds from our employee stock purchase plan ("ESPP") of \$0.1 million. Net cash provided by financing activities during 2016 consists of proceeds from the exercise of stock options and warrants of \$2.4 million and proceeds from our ESPP of \$0.3 million. Net cash provided by financing activities during 2015 consists of net proceeds from the issuance of common stock from our 2015 public offerings of common stock totaling \$143.3 million, proceeds from exercise of common stock options and warrants of \$0.6 million and proceeds from our ESPP of \$0.5 million.

## Contractual Obligations and Commitments

The following table summarizes our contractual obligations and commitments as of December 31, 2017 that will affect our future liquidity (in thousands):

|                                            |       | Less  |       |       | More  |   |
|--------------------------------------------|-------|-------|-------|-------|-------|---|
|                                            | Total | Than  | 1 -3  | 3 -5  | Than  |   |
|                                            |       | 1     | Years | Years | 5     |   |
|                                            |       | year  |       |       | Years |   |
| Operating lease obligations <sup>(1)</sup> | \$315 | \$289 | \$26  | \$    | —\$   | — |
| Total Contractual Obligations              | \$315 | \$289 | \$26  | \$    | —\$   | — |

<sup>(1)</sup> In June 2014 we entered into a multi-year non-cancelable building lease for office space in San Diego, California which expired in January 2018. In March 2017, we amended the lease to extend the term through January 2019.

We enter into contracts in the normal course of business with clinical sites for the conduct of clinical trials, CROs for clinical research studies, professional consultants for expert advice and other vendors for clinical supply manufacturing or other services. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

## Off-Balance Sheet Arrangements

During the years ended December 31, 2017 and 2016, we did not have any off-balance sheet arrangements (as defined by applicable SEC regulations) that are reasonably likely to have a current or future material effect on our financial condition, results of operations, liquidity, capital expenditures or capital resources.

## JOBS Act

In April 2012, the JOBS Act was enacted. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other companies.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, as an “emerging growth company,” we intend to rely on certain of these exemptions, including without limitation with respect to, (1) providing an auditor’s attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (2) complying with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements, known as the auditor discussion and analysis. We expect that we will remain an emerging growth company until December 31, 2018.

## Item 7A. Quantitative and Qualitative Disclosures about Market Risk

Some of our short-term investments have market risk in that a change in prevailing interest rates may cause the principal amount of the investment to fluctuate. Financial instruments that potentially subject us to significant concentrations of credit risk consist primarily of cash, cash equivalents and short-term investments. We invest our

excess cash primarily in commercial paper and debt instruments of financial institutions, corporations, U.S. government-sponsored agencies and the U.S. Treasury. We mitigate credit risk by maintaining a well-diversified portfolio and limiting the amount of investment exposure as to institution, maturity and investment type. We invest our excess cash in accordance with our investment policy.

Because of the short-term maturities of our cash equivalents and short-term investments, we do not believe that an increase in market rates would have any significant impact on the realized value of our investments. If a 1% change in interest rates were to have occurred on December 31, 2017, this change would not have had a material effect on the fair value of our investment portfolio as of that date.

Item 8. Financial Statements and Supplementary Data

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

Item 9. Changes In and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

As required by Rule 13a-15(b) and Rule 15d-15(b) of the Exchange Act, our management, including our principal executive officer and our principal financial officer, conducted an evaluation as of the end of the period covered by this Annual Report on Form 10-K of the effectiveness of the design and operation of our disclosure controls and procedures. Based on that evaluation, management has concluded that as of December 31, 2017, the Company's disclosure controls and procedures were effective at the reasonable assurance level and we believe the consolidated financial statements included in this Form 10-K for the year ended December 31, 2017 present, in all material respects, our financial position, results of operations, comprehensive loss and cash flows for the periods presented in conformity with U.S. generally accepted accounting principles.

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). Internal control over financial reporting is a process designed under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States of America.

As of December 31, 2017, our management assessed the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control-Integrated Framework (2013 Framework). Based on this assessment, our management concluded that, as of December 31, 2017, our internal control over financial reporting was effective based on those criteria.

This Annual Report on Form 10-K does not include an attestation report of our registered public accounting firm due to a transition period established by the JOBS Act for emerging growth companies.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting identified in management's evaluation pursuant to Rules 13a-15(d) or 15d-15(d) of the Exchange Act during the quarter ended December 31, 2017 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

Recently Adopted Accounting Pronouncements



See “Notes to Financial Statements-Note 3-Recently Issued and Recently Adopted Accounting Pronouncements” of our annual financial statements.

### PART III

#### Item 10. Directors, Executive Officers and Corporate Governance

The information required by this item with respect to directors is incorporated by reference from the information under the captions "Election of Directors," "Section 16(a) Beneficial Ownership Reporting Compliance," and "Code of Ethics" contained in the proxy statement to be filed with the SEC pursuant to Regulation 14A in connection with our 2018 annual meeting of stockholders.

#### Item 11. Executive Compensation

The information required by this item is incorporated by reference to the information under the captions "Non-Employee Director Compensation," "Executive Compensation" and "Compensation Committee Interlocks and Insider Participation" contained in the proxy statement to be filed with the SEC pursuant to Regulation 14A in connection with our 2018 annual meeting of stockholders.

#### Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item is incorporated by reference to the information under the captions "Security Ownership of Certain Beneficial Owners and Management" and "Equity Compensation Plan Information" contained in the proxy statement to be filed with the SEC pursuant to Regulation 14A in connection with our 2018 annual meeting of stockholders.

#### Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this item is incorporated by reference to the information under the captions "Election of Directors" and "Certain Relationships and Related Transactions" contained in the proxy statement to be filed with the SEC pursuant to Regulation 14A in connection with our 2018 annual meeting of stockholders.

#### Item 14. Principal Accountant Fees and Services

The information required by this item is incorporated by reference to the information under the caption contained in "Ratification of Selection of Independent Registered Public Accounting Firm" contained in the proxy statement to be filed with the SEC pursuant to Regulation 14A in connection with our 2018 annual meeting of stockholders.

PART IV

Item 15. Exhibits and Financial Statement Schedules

1. Financial Statements. We have filed the following documents as part of this Annual Report:

|                                                                     | Page      |
|---------------------------------------------------------------------|-----------|
| Consolidated Financial Statements                                   |           |
| <u>Report of Independent Registered Public Accounting Firm</u>      | <u>55</u> |
| Financial Statements:                                               |           |
| <u>Consolidated Balance Sheets</u>                                  | <u>56</u> |
| <u>Consolidated Statements of Operations and Comprehensive Loss</u> | <u>57</u> |
| <u>Consolidated Statements of Changes in Stockholders' Equity</u>   | <u>58</u> |
| <u>Consolidated Statements of Cash Flows</u>                        | <u>59</u> |
| <u>Notes to Consolidated Financial Statements</u>                   | <u>60</u> |

2. Financial Statement Schedules. All schedules are omitted because they are not applicable or the required information is shown in the Financial Statements or notes thereto.

## INDEX TO EXHIBITS

| Exhibit number | Description of document                                                                                                                                                                                    |
|----------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| 2.1            | <u>Arrangement Agreement, dated May 8, 2013, by and between MethylGene Inc. and the Registrant.</u> <sup>(2)</sup>                                                                                         |
| 3.1            | <u>Amended and Restated Certificate of Incorporation.</u> <sup>(1)</sup>                                                                                                                                   |
| 3.2            | <u>Bylaws.</u> <sup>(1)</sup>                                                                                                                                                                              |
| 3.3            | <u>Amendment to Bylaws.</u> <sup>(7)</sup>                                                                                                                                                                 |
| 4.1            | <u>Form of Common Stock Certificate.</u> <sup>(2)</sup>                                                                                                                                                    |
| 4.2            | <u>Form of Warrant to Purchase Common Stock.</u> <sup>(9)</sup>                                                                                                                                            |
| 4.3            | <u>Form of Warrant to Purchase Common Stock</u> <sup>(12)</sup>                                                                                                                                            |
| 10.1           | <u>Form of Securities Purchase Agreement relating to the 2011 private placement.</u> <sup>(1)</sup>                                                                                                        |
| 10.2           | <u>Form of Securities Purchase Agreement relating to the 2012 private placement.</u> <sup>(1)</sup>                                                                                                        |
| 10.3+          | <u>Amended and Restated Incentive Stock Option Plan.</u> <sup>(1)</sup>                                                                                                                                    |
| 10.4+          | <u>Amended and Restated 2013 Equity Incentive Plan and Form of 2013 Equity Incentive Plan and Form of Stock Option Grant Notice and Form of Stock Option Agreement thereunder.</u> <sup>(8)</sup>          |
| 10.5+          | <u>Form of 2013 Employee Stock Purchase Plan.</u> <sup>(1)</sup>                                                                                                                                           |
| 10.6*          | <u>Collaboration and License Agreement, dated October 16, 2003, by and between MethylGene Inc. and Taiho Pharmaceutical Co. Ltd.</u> <sup>(1)</sup>                                                        |
| 10.7*          | <u>Amendment Number One to Collaboration and License Agreement, dated January 25, 2005, by and between MethylGene Inc. and Taiho Pharmaceutical Co., Ltd.</u> <sup>(1)</sup>                               |
| 10.8*          | <u>Letter Agreement, dated January 25, 2005, by and between MethylGene Inc. and Taiho Pharmaceutical Co., Ltd., relating to Collaboration and License Agreement dated October 16, 2003.</u> <sup>(1)</sup> |
| 10.9+          | <u>Senior Executive Employment Agreement, dated September 24, 2012, by and among MethylGene Inc. and Dr. Charles M. Baum.</u> <sup>(1)</sup>                                                               |
| 10.10+         | <u>Amended and Restated Employment Agreement, dated July 2, 2013, by and between the Registrant and Dr. Charles M. Baum.</u> <sup>(3)</sup>                                                                |
| 10.11          | <u>Lease Agreement, dated June 24, 2014, by and between the Company and ARE-SD Region No. 20, LLC.</u> <sup>(5)</sup>                                                                                      |
| 10.12          | <u>First Amendment to Lease to 9393 Towne Centre Drive, dated March 23, 2017.</u> <sup>(10)</sup>                                                                                                          |
| 10.13+         | <u>Letter Agreement, dated August 30, 2013, by and between the Registrant and Dr. Isan Chen.</u> <sup>(4)</sup>                                                                                            |
| 10.14+         | <u>Letter Agreement, dated May 20, 2013, by and between Methylgene Inc. and James Christensen.</u> <sup>(6)</sup>                                                                                          |
| 10.15+         | <u>Form of Indemnity Agreement.</u> <sup>(4)</sup>                                                                                                                                                         |
| 10.16+         | <u>Amended and Restated Non-Employee Director Compensation Policy.</u>                                                                                                                                     |
| 10.17+         | <u>Letter Agreement, dated September 13, 2016, by and between Mirati Therapeutics, Inc. and Christopher LeMasters.</u> <sup>(11)</sup>                                                                     |
| 10.18+         | <u>Amendment to Amended and Restated Employment Agreement, dated December 19, 2016, by and between the Registrant and Dr. Charles Baum.</u> <sup>(11)</sup>                                                |
| 10.19+         | <u>Amendment to Letter Agreement, dated December 19, 2016, by and between the Registrant and Jamie Donadio.</u> <sup>(11)</sup>                                                                            |
| 10.20+         | <u>Amendment to Letter Agreement, dated December 19, 2016, by and between the Registrant and Dr. Isan Chen.</u> <sup>(11)</sup>                                                                            |
| 10.21+         | <u>Amendment to Letter Agreement, dated December 19, 2016, by and between the Registrant and James Christensen.</u> <sup>(11)</sup>                                                                        |
| 10.22+         | <u>Amendment to Letter Agreement, dated December 19, 2016, by and between the Registrant and Christopher LeMasters.</u> <sup>(11)</sup>                                                                    |
| 21.1           | <u>Subsidiaries of the Registrant.</u> <sup>(1)</sup>                                                                                                                                                      |
| 23.1           | <u>Consent of Independent Registered Public Accounting Firm-US.</u>                                                                                                                                        |
| 31.1           |                                                                                                                                                                                                            |

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Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.

31.2 Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.

32.1 Certifications Pursuant to U.S.C. Section 1350, As Adopted Pursuant to Section 906 of the Public Company Accounting Reform and Investor Protection Act of 2002.

101.INS XBRL Instance Document.

101.SCH XBRL Taxonomy Extension Schema Document.

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101.CALXBRL Taxonomy Extension Schema Document.

101.DEF XBRL Taxonomy Extension Definition Linkbase Document.

101.LAB XBRL Taxonomy Extension Label Linkbase Document.

101.PRE XBRL Taxonomy Extension Presentation Linkbase Document.

+ Indicates management contract or compensatory plan.

\* We have received confidential treatment for certain portions of this agreement, which have been omitted and filed separately with the SEC pursuant to Rule 406 under the Securities Act.

- (1) Incorporated by reference to Mirati Therapeutics, Inc.'s Registration Statement on Form 10-12B (No. 001-35921), filed with the Securities and Exchange Commission on May 10, 2013.
- (2) Incorporated by reference to Mirati Therapeutics, Inc.'s Amended Registration Statement on Form 10-12B/A (No. 001-35921), filed with the Securities and Exchange Commission on June 14, 2013.
- (3) Incorporated by reference to Mirati Therapeutics, Inc.'s Amended Registration Statement on Form 10-12B/A (No. 001-35921), filed with the Securities and Exchange Commission on July 9, 2013.
- (4) Incorporated by reference to Mirati Therapeutics, Inc.'s Registration Statement on Form S-1 (No. 333-191544), filed with the Securities and Exchange Commission on October 3, 2013.
- (5) Incorporated by reference to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on June 27, 2014.
- (6) Incorporated by reference to Mirati Therapeutics, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2014, filed with the Securities and Exchange Commission on March 11, 2015.
- (7) Incorporated by reference to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on June 16, 2016.
- (8) Incorporated by reference to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on May 19, 2017.
- (9) Incorporated by reference to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on January 6, 2017.
- (10) Incorporated by reference to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on March 27, 2017.
- (11) Incorporated by reference to Mirati Therapeutics, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2016, filed with the Securities and Exchange Commission on March 9, 2017.
- (12) Incorporated by reference to Mirati Therapeutics, Inc.'s Current Report on Form 8-K, filed with the Securities and Exchange Commission on November 16, 2017.

#### Item 16. Form 10-K Summary

None

## SIGNATURES

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

### MIRATI THERAPEUTICS, INC.

Date: March 8, 2018 by: /s/ Charles M. Baum  
President and Chief Executive Officer  
(Principal Executive Officer)

Date: March 8, 2018 by: /s/ Jamie A. Donadio  
Senior Vice President and Chief Financial Officer  
(Principal Financial and Accounting Officer)

## POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Charles M. Baum, Ph.D. and Jamie A. Donadio as his or her true and lawful attorneys-in-fact, and each of them, with full power of substitution, for him or her in any and all capacities, to sign any amendments to this Annual Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in and about the premises, as fully to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact, and either of them, or his or their substitute or substitutes may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this Annual Report on Form 10-K 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/ CHARLES M. BAUM<br>Charles M. Baum, M.D.,<br>Ph.D. | President, Chief Executive Officer and Director (Principal Executive Officer)                     | March 8, 2018 |
| /S/ JAMIE A. DONADIO<br>Jamie A. Donadio               | Senior Vice President and Chief Financial Officer<br>(Principal Financial and Accounting Officer) | March 8, 2018 |
| /S/ RODNEY LAPPE<br>Rodney Lappe, Ph.D.                | Chairman of the Board                                                                             | March 8, 2018 |
| /S/ BRUCE L.A. CARTER<br>Bruce L.A. Carter, Ph.D.      | Director                                                                                          | March 8, 2018 |
| /S/ HENRY J. FUCHS<br>Henry J. Fuchs, M.D.             | Director                                                                                          | March 8, 2018 |
| /S/ MICHAEL GREY<br>Michael Grey                       | Director                                                                                          | March 8, 2018 |
| /S/ CRAIG JOHNSON<br>Craig Johnson                     | Director                                                                                          | March 8, 2018 |
| /S/ NEIL A. REISMAN<br>Neil A. Reisman                 | Director                                                                                          | March 8, 2018 |



Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Mirati Therapeutics, Inc.

Opinion on the Financial Statements

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

Basis for Opinion

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

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

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

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2014

San Diego, California

March 8, 2018

Mirati Therapeutics, Inc.

## CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share data)

|                                                                                                                                                                    | December 31, |           |
|--------------------------------------------------------------------------------------------------------------------------------------------------------------------|--------------|-----------|
|                                                                                                                                                                    | 2017         | 2016      |
| <b>ASSETS</b>                                                                                                                                                      |              |           |
| Current assets                                                                                                                                                     |              |           |
| Cash and cash equivalents                                                                                                                                          | \$ 107,703   | \$ 22,383 |
| Short-term investments                                                                                                                                             | 43,134       | 34,351    |
| Other current assets                                                                                                                                               | 4,922        | 2,821     |
| Total current assets                                                                                                                                               | 155,759      | 59,555    |
| Property and equipment, net                                                                                                                                        | 525          | 629       |
| Other long-term assets                                                                                                                                             | 962          | 3,260     |
| Total assets                                                                                                                                                       | \$ 157,246   | \$ 63,444 |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>                                                                                                                        |              |           |
| Current liabilities                                                                                                                                                |              |           |
| Accounts payable and accrued liabilities                                                                                                                           | \$ 13,644    | \$ 15,002 |
| Total current liabilities                                                                                                                                          | 13,644       | 15,002    |
| Other liabilities                                                                                                                                                  | 314          | 133       |
| Total liabilities                                                                                                                                                  | 13,958       | 15,135    |
| Stockholders' equity                                                                                                                                               |              |           |
| Preferred stock, \$0.001 par value, 10,000,000 shares authorized; none issued and outstanding at both December 31, 2017 and December 31, 2016                      | —            | —         |
| Common stock, \$0.001 par value; 100,000,000 authorized; 28,622,886 and 19,937,095 issued and outstanding at December 31, 2017 and December 31, 2016, respectively | 29           | 20        |
| Additional paid-in capital                                                                                                                                         | 594,407      | 428,507   |
| Accumulated other comprehensive income                                                                                                                             | 9,479        | 9,533     |
| Accumulated deficit                                                                                                                                                | (460,627 )   | (389,751) |
| Total stockholders' equity                                                                                                                                         | 143,288      | 48,309    |
| Total liabilities and stockholders' equity                                                                                                                         | \$ 157,246   | \$ 63,444 |

See accompanying notes

Mirati Therapeutics, Inc.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except share and per share data)

|                                                               | Year Ended December 31, |             |             |
|---------------------------------------------------------------|-------------------------|-------------|-------------|
|                                                               | 2017                    | 2016        | 2015        |
| Expenses                                                      |                         |             |             |
| Research and development                                      | \$58,085                | \$68,487    | \$48,959    |
| General and administrative                                    | 13,450                  | 15,292      | 15,755      |
| Total operating expenses                                      | 71,535                  | 83,779      | 64,714      |
| Loss from operations                                          | (71,535 )               | (83,779 )   | (64,714 )   |
| Other income, net                                             | 1,105                   | 661         | 170         |
| Net loss                                                      | \$(70,430)              | \$(83,118 ) | \$(64,544 ) |
| Unrealized gain (loss) on available-for-sale investments      | \$(54 )                 | \$(25 )     | \$37        |
| Comprehensive loss                                            | \$(70,484)              | \$(83,143 ) | \$(64,507 ) |
| Basic and diluted net loss per share                          | \$(2.78 )               | \$(4.20 )   | \$(3.82 )   |
| Weighted average common shares outstanding, basic and diluted | 25,290,222              | 19,787,349  | 16,901,826  |

See accompanying notes

Mirati Therapeutics, Inc.

## CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

(in thousands, except share data)

|                                                                        | Common Stock |        | Additional<br>paid-in<br>capital | Accumulated<br>other<br>comprehensive<br>income<br>(loss) | Accumulated<br>deficit | Total<br>stockholders'<br>equity |
|------------------------------------------------------------------------|--------------|--------|----------------------------------|-----------------------------------------------------------|------------------------|----------------------------------|
|                                                                        | Shares       | Amount |                                  |                                                           |                        |                                  |
| Balance at January 1, 2014                                             | 13,566,726   | \$ 14  | \$ 260,616                       | \$ 9,521                                                  | \$ (242,089 )          | \$ 28,062                        |
| Net loss for the year                                                  | —            | —      | —                                | —                                                         | (64,544 )              | (64,544 )                        |
| Share-based compensation expense                                       | —            | —      | 10,254                           | —                                                         | —                      | 10,254                           |
| Issuance of common stock, net of costs                                 | 4,837,500    | 4      | 143,289                          | —                                                         | —                      | 143,293                          |
| Issuance of common stock from Employee Stock Purchase Plan ("ESPP")    | 32,645       | —      | 522                              | —                                                         | —                      | 522                              |
| Exercise of options for cash                                           | 36,566       | —      | 552                              | —                                                         | —                      | 552                              |
| Net exercise of warrants                                               | 809,498      | 1      | (1 )                             | —                                                         | —                      | —                                |
| Unrealized gain on investments                                         | —            | —      | —                                | 37                                                        | —                      | 37                               |
| Balance at December 31, 2015                                           | 19,282,935   | \$ 19  | \$ 415,232                       | \$ 9,558                                                  | \$ (306,633 )          | \$ 118,176                       |
| Net loss for the year                                                  | —            | —      | —                                | —                                                         | (83,118 )              | (83,118 )                        |
| Share-based compensation expense                                       | —            | —      | 10,624                           | —                                                         | —                      | 10,624                           |
| Issuance of common stock from ESPP                                     | 28,483       | —      | 297                              | —                                                         | —                      | 297                              |
| Exercise of options for cash                                           | 22,132       | —      | 240                              | —                                                         | —                      | 240                              |
| Exercise of warrants for cash                                          | 313,756      | 1      | 2,114                            | —                                                         | —                      | 2,115                            |
| Net exercise of warrants                                               | 289,789      | —      | —                                | —                                                         | —                      | —                                |
| Unrealized loss on investments                                         | —            | —      | —                                | (25 )                                                     | —                      | (25 )                            |
| Balance at December 31, 2016                                           | 19,937,095   | \$ 20  | \$ 428,507                       | \$ 9,533                                                  | \$ (389,751 )          | \$ 48,309                        |
| Net loss for the year                                                  | —            | —      | —                                | —                                                         | (70,430 )              | (70,430 )                        |
| Issuance of common stock and warrants, net of costs                    | 7,941,688    | 8      | 153,522                          | —                                                         | —                      | 153,530                          |
| Share-based compensation expense                                       | —            | —      | 6,786                            | —                                                         | —                      | 6,786                            |
| Cumulative effect of accounting change for the adoption of ASU 2016.09 | —            | —      | 446                              | —                                                         | (446 )                 | —                                |
| Issuance of common stock from ESPP                                     | 59,976       | —      | 144                              | —                                                         | —                      | 144                              |
| Exercise of options for cash                                           | 45,573       | —      | 399                              | —                                                         | —                      | 399                              |
| Exercise of warrants for cash                                          | 585,729      | 1      | 4,603                            | —                                                         | —                      | 4,604                            |
| Net exercise of warrants                                               | 52,825       | —      | —                                | —                                                         | —                      | —                                |
| Unrealized loss on investments                                         | —            | —      | —                                | (54 )                                                     | —                      | (54 )                            |
| Balance at December 31, 2017                                           | 28,622,886   | \$ 29  | \$ 594,407                       | \$ 9,479                                                  | \$ (460,627 )          | \$ 143,288                       |

See accompanying notes

## Mirati Therapeutics, Inc.

## CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

|                                                                   | Years Ended December 31, |             |             |
|-------------------------------------------------------------------|--------------------------|-------------|-------------|
|                                                                   | 2017                     | 2016        | 2015        |
| Operating activities:                                             |                          |             |             |
| Net loss                                                          | \$(70,430 )              | \$(83,118 ) | \$(64,544 ) |
| Non-cash adjustments reconciling net loss to operating cash flows |                          |             |             |
| Depreciation of property and equipment                            | 184                      | 180         | 212         |
| Amortization of premium on investments                            | (266 )                   | 7           | 337         |
| Share-based compensation expense                                  | 6,786                    | 10,624      | 10,254      |
| Changes in operating assets and liabilities                       |                          |             |             |
| Other current assets                                              | (3,409 )                 | 254         | 279         |
| Other long-term assets                                            | 3,606                    | (1,259 )    | (1,675 )    |
| Accounts payable and accrued liabilities                          | (1,341 )                 | 5,196       | 4,370       |
| Other current and long term liabilities                           | 164                      | 99          | 53          |
| Cash flows used in operating activities                           | (64,706 )                | (68,017 )   | (50,714 )   |
| Investing activities:                                             |                          |             |             |
| Purchases of short-term investments                               | (100,558 )               | (70,269 )   | (104,954 )  |
| Sales and maturities of short-term investments                    | 91,988                   | 108,720     | 54,530      |
| Purchases of property and equipment                               | (81 )                    | (196 )      | (329 )      |
| Cash flows provided by (used in) investing activities             | (8,651 )                 | 38,255      | (50,753 )   |
| Financing activities:                                             |                          |             |             |
| Proceeds from issuance of common stock, net of issuance costs     | 153,530                  | —           | 143,293     |
| Proceeds from exercise of common stock options and warrants       | 5,003                    | 2,355       | 552         |
| Proceeds from issuance under employee stock purchase plan         | 144                      | 297         | 522         |
| Cash flows provided by financing activities                       | 158,677                  | 2,652       | 144,367     |
| Increase (decrease) in cash and cash equivalents                  | 85,320                   | (27,110 )   | 42,900      |
| Cash and cash equivalents, beginning of year                      | 22,383                   | 49,493      | 6,593       |
| Cash and cash equivalents, end of year                            | \$107,703                | \$22,383    | \$49,493    |

See accompanying notes

Mirati Therapeutics, Inc.  
Notes to Consolidated Financial Statements

1. Description of Business

Mirati Therapeutics, Inc. ("Mirati" or the "Company") is a clinical-stage oncology company developing targeted drug products to address the genetic, epigenetic and immunological promoters of cancer.

The Company's common stock has been listed on the NASDAQ Capital Market since July 15, 2013 under the ticker symbol "MRTX." The Company has a wholly owned subsidiary in Canada, MethylGene, Inc. ("MethylGene").

2. Summary of Significant Accounting Policies

Basis of Presentation

These consolidated financial statements are prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"). These consolidated financial statements include the accounts of the Company and MethylGene. All significant inter-company transactions, balances and expenses have been eliminated upon consolidation.

Mirati was incorporated under the laws of the State of Delaware on April 29, 2013. On May 8, 2013, the Company's Board of Directors approved and the Company entered into an arrangement agreement ("Arrangement") with MethylGene. Upon completion of the Arrangement, MethylGene became the Company's wholly-owned subsidiary.

These consolidated financial statements are presented in United States ("U.S.") Dollars, which is also the functional currency of the Company.

Use of Estimates

The preparation of the Company's audited consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period.

Reported amounts and note disclosures reflect the overall economic conditions that are most likely to occur and anticipated measures management intends to take. Actual results could differ materially from those estimates. Estimates and assumptions are reviewed quarterly. Any revisions to accounting estimates are recognized in the period in which the estimates are revised and in any future periods affected.

Cash, Cash Equivalents and Short-term Investments

Cash and cash equivalents consist of cash and highly liquid securities with original maturities at the date of acquisition of ninety days or less. Investments with an original maturity of more than ninety days are considered short-term investments and have been classified by management as available-for-sale. These investments are classified as current assets, even though the stated maturity date may be one year or more beyond the current balance sheet date, which reflects management's intention to use the proceeds from sales of these securities to fund its operations, as necessary. Such investments are carried at fair value, with unrealized gains and losses included as a separate component of stockholders' equity. Realized gains and losses from the sale of available-for-sale securities or the amounts, net of tax,

reclassified out of accumulated other comprehensive income, if any, are determined on a specific identification basis.

## Concentration of Credit Risk

The Company invests its excess cash in accordance with its investment policy. The Company's investments are comprised primarily of commercial paper and debt instruments of financial institutions, corporations, U.S. government-sponsored agencies and the U.S. Treasury. The Company mitigates credit risk by maintaining a diversified portfolio and limiting the amount of investment exposure as to institution, maturity and investment type. Financial instruments that potentially subject the Company to significant credit risk consist principally of cash equivalents and short-term investments.

## Property and Equipment, Net

Property and equipment is stated at historical cost less accumulated depreciation. Historical cost includes expenditures that are directly attributable to the acquisition of the items. All repairs and maintenance are charged to net loss during the financial period in which they are incurred.

Depreciation of property and equipment is calculated using the straight-line method over the estimated useful lives of the assets, as follows:

|                            |                                                       |
|----------------------------|-------------------------------------------------------|
| Computer equipment         | 3 years                                               |
| Office and other equipment | 6 years                                               |
| Laboratory equipment       | 6 years                                               |
| Leasehold improvements     | The lesser of the lease term or the life of the asset |

Upon disposal or impairment of property and equipment, the cost and related accumulated depreciation is removed from the consolidated financial statements and the net amount, less any proceeds, is included in net loss.

## Impairment of Long-Lived Assets

The Company reviews its long-lived assets for impairment whenever events or changes in circumstances indicate that the carrying value of an asset 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. Fair value is estimated through discounted cash flow models to project cash flows from the asset. The Company recognized no impairment charges for the years ended December 31, 2017, 2016 and 2015.

## Share-Based Compensation

The Company measures and recognizes compensation expense for share-based payments based on estimated fair value, using the fair value of stock options granted using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of certain estimates and highly judgmental assumptions that affect the amount of share-based compensation expense recognized in the Company's consolidated financial statements. These assumptions include the historical volatility of the Company's stock price, expected term of the options, the risk-free interest rate and expected dividend yields. Share-based compensation is recognized using the graded accelerated vesting method. If any of the assumptions used in the Company's calculation change significantly, share-based compensation expense may differ materially from what the Company has recorded in the current period.





### Investment Tax Credits

The Company's accounts include claims for investment tax credits ("ITCs") relating to scientific research and experimental development activities of the Company. The qualification and recording of these activities for investment tax credit purposes are established by the Canadian federal and Provincial Tax Acts and are subject to audit by the taxation authorities. Refundable ITCs are reflected as reductions of expenses or reductions of the cost of the assets to which they relate when there is reasonable assurance that the assistance will be received and all conditions have been complied with. The non-refundable ITCs are carried forward for a time and will be recognized when it is more likely than not that the Company will become subject to Canadian federal taxes, at which time, these ITCs will be applied as a reduction of tax expense. As operations in Canada ceased in early 2014, there were no new ITCs earned for the years ended December 31, 2017, 2016 or 2015.

### Research and Development Expenses

Research and development expenditures are charged to net loss in the period in which they are incurred and are comprised of the following types of costs incurred in performing research and development activities: contract services for clinical trials and related clinical manufacturing costs, salaries and benefits including share-based compensation expense, costs for allocated facilities and depreciation of equipment and license fees paid in connection with our early discovery efforts.

### 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 applicable 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 net loss 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 consolidated financial statements.

### Segment Reporting

Operating segments are components of a business where separate discrete financial information is available for evaluation by the chief operating decision-maker for purposes of making decisions regarding resource allocation and assessing performance. To date, the Company and the chief operating decision-maker has viewed its operations and managed its business as one segment operating primarily in the United States.

### Net Loss per Share

Basic net loss per common share is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding during the period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common shares and potentially dilutive securities outstanding for the period.

Common share equivalents outstanding, determined using the treasury stock method, are comprised of shares that may be issued under the Company's stock option and warrant agreements.

The following table presents the weighted average number of potentially dilutive securities not included in the calculation of diluted net loss per share due to the anti-dilutive effect of the securities:

|                       | Year ended<br>December 31, |         |           |
|-----------------------|----------------------------|---------|-----------|
|                       | 2017                       | 2016    | 2015      |
| Common stock options  | 38,675                     | 173,776 | 582,662   |
| Common stock warrants | 7,534,576                  | 315,834 | 1,546,201 |
| Total                 | 7,573,251                  | 489,610 | 2,128,863 |

### 3. Recently Issued and Recently Adopted 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 the Company as of the specified effective date.

#### Recently Adopted Accounting Pronouncements

In January 2017, the FASB issued ASU No. 2017-01, Clarifying the Definition of a Business, which clarifies and provides a more robust framework to use in determining when a set of assets and activities is a business. The amendments in this update should be applied prospectively on or after the effective date. This update is effective for annual periods beginning after December 15, 2017, and interim periods within those periods. Early adoption is permitted for acquisition or deconsolidation transactions occurring before the issuance date or effective date and only when the transactions have not been reported in issued financial statements. The Company early adopted this standard effective April 1, 2017 in connection with an immaterial collaboration agreement it entered into during the quarter. The adoption of this standard did not have a material effect on its consolidated financial statements.

In March 2016, the FASB issued Accounting Standard Update ("ASU") 2016-09, Compensation-Stock Compensation (Topic 718). The new guidance changes the accounting and simplifies various aspects of the accounting for share-based payments to employees. The guidance allows for a policy election to account for forfeitures as they occur or based on an estimated number of awards that are expected to vest. ASU 2016-09 is effective for annual periods beginning after December 15, 2016, with early adoption permitted. Effective January 1, 2017, the Company adopted the provisions of ASU 2016-09. The impact of this adoption was limited to the accounting for forfeitures of certain stock based awards, which is adopted on a modified retrospective basis. Upon adoption, the Company will no longer estimate forfeitures and will instead account for forfeitures as they occur. This policy election was made to allow simplification of the accounting for share-based awards. The cumulative effect of adoption was an increase to both additional paid-in capital and accumulated deficit of \$0.4 million.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements-Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern. Under the new guidance, management is required to assess an entity's ability to continue as a going concern, and to provide related footnote disclosures in certain circumstances. The provisions of this ASU are effective for annual periods ending after December 15, 2016, and for annual and interim periods thereafter; early adoption is permitted. We adopted this guidance as of December 31, 2016 and the adoption did not require any additional disclosures in our consolidated financial statements for the year ended December 31, 2016 or for the current period.

In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which will replace numerous requirements in U.S. GAAP, including industry-specific requirements, and provide companies with a single revenue recognition model for recognizing revenue from contracts with customers. The core principle of the new standard is that a company should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. In August 2015, the FASB approved a proposal to defer the effective date of the guidance until annual and interim reporting periods beginning after December 15, 2017. Although we currently do not have any revenue contracts in 2017, we early adopted this standard effective January 1, 2017 using the full retrospective method of adoption so that, in the event we enter into any revenue contracts, the contracts will be accounted for under the new guidance from inception of the contract.

#### Recently Issued Accounting Pronouncements

In July 2017, the FASB issued ASU 2017-11, Earnings Per Share (Topic 260); Distinguishing Liabilities from Equity (Topic 480); Derivatives and Hedging (Topic 815): (Part I) Accounting for Certain Financial Instruments with Down Round Features, (Part II) Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Non-controlling Interests with a Scope Exception. The ASU allows companies to exclude a down round feature when determining whether a financial instrument (or embedded conversion feature) is considered indexed to the entity's own stock. As a result, financial instruments (or embedded conversion features) with down round features may no longer be required to be accounted classified as liabilities. A company will recognize the value of a down round feature only when it is triggered and the strike price has been adjusted downward. For equity-classified freestanding financial instruments, such as warrants, an entity will treat the value of the effect of the down round, when triggered, as a dividend and a reduction of income available to common shareholders in computing basic earnings per share. For convertible instruments with embedded conversion features containing down round provisions, entities will recognize the value of the down round as a beneficial conversion discount to be amortized to earnings. The guidance in ASU 2017-11 is effective for fiscal years beginning after December 15,

2018, and interim periods within those fiscal years. Early adoption is permitted, and the guidance is to be applied using a full or modified retrospective approach. The Company does not anticipate that the adoption of ASU 2017-11 will have a material impact on its consolidated financial statements unless a transaction occurs that would need to be evaluated under this guidance at which time the Company will assess the impact of this standard.

In May 2017, the FASB issued ASU 2017-09, Compensation-Stock Compensation, to provide clarity and reduce both 1) diversity in practice and 2) cost and complexity when applying the guidance in Topic 718 to a change in the terms or conditions of a share-based payment award. ASU 2017-09 provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting under Topic 718. The amendments in ASU 2017-09 are effective for fiscal and interim reporting periods in fiscal years beginning after December 15, 2017. Early adoption is permitted, including adoption in any interim period. The amendments in ASU 2017-09 should be applied prospectively to an award modified on or after the adoption date. The Company does not anticipate that the adoption of ASU 2017-09 will have a material impact on its consolidated financial statements unless a transaction occurs that would need to be evaluated under this guidance at which time the Company will assess the impact of this standard.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842) in order to increase transparency and comparability among organizations by recognizing lease assets and lease liabilities on the balance sheet for those leases classified as operating leases under previous GAAP. ASU 2016-02 requires that a lessee should recognize a liability to make lease payments (the lease liability) and a right-of-use asset representing its right to use the underlying asset for the lease term on the balance sheet. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018 (including interim periods within those periods) using a modified retrospective approach and early adoption is permitted. The Company will adopt ASU 2016-02 in the first quarter of 2019. Although the Company is in the process of evaluating the impact of adoption of the ASU on its consolidated financial statements, the Company currently believes the most significant changes will be related to the recognition of new right-of-use assets and lease liabilities on the Company's balance sheet for real estate operating leases.

In January 2016, the FASB issued ASU No. 2016-01, Financial Instruments - Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities. The new guidance enhances the reporting model for financial instruments and includes amendments to address aspects of recognition, measurement, presentation and disclosure. The update to the standard is effective for public companies for interim and annual periods beginning after December 15, 2017. The Company does not believe the adoption of this standard will have a material impact on its financial position, results of operations or related financial statement disclosures.

#### 4. Investments

The following tables summarize our short-term investments (in thousands):

| As of December 31, 2017 |                |                        |                         |                      |
|-------------------------|----------------|------------------------|-------------------------|----------------------|
| Maturity                | Amortized cost | Gross unrealized gains | Gross unrealized losses | Estimated fair value |
| Corporate debt or less  | \$24,264       | \$ —                   | \$ (23 )                | \$ 24,241            |
| Equity securities       |                |                        |                         |                      |
| Commercial paper        | 18,912         | —                      | (19 )                   | 18,893               |
|                         | \$43,176       | \$ —                   | \$ (42 )                | \$ 43,134            |
| As of December 31, 2016 |                |                        |                         |                      |
| Maturity                |                |                        |                         |                      |

|                                         | Amortized<br>cost | Gross<br>unrealized<br>gains | Gross<br>unrealized<br>losses | Estimated<br>fair value |
|-----------------------------------------|-------------------|------------------------------|-------------------------------|-------------------------|
| Corporate<br>debt or less<br>securities | \$20,622          | \$ —                         | \$ (3 )                       | \$ 20,619               |
| Commercial<br>1 year or less<br>paper   | 13,717            | 15                           | —                             | 13,732                  |
|                                         | \$34,339          | \$ 15                        | \$ (3 )                       | \$ 34,351               |

Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At December 31, 2017, the Company did not have any securities in material unrealized loss positions. The Company reviews its investments to identify and evaluate investments that have an indication of possible other-than-temporary impairment. Factors considered in determining whether a loss is other-than-temporary include the length of time and extent to which fair value has been less than the cost basis, the financial condition and near-term prospects of the investee, and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value. The Company does not intend to sell any investments prior to recovery of their amortized cost basis for any investments in an unrealized loss position.

## 5. Fair Value Measurements

The Company has certain financial assets and liabilities recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

The authoritative guidance for fair value measurements defines fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or the most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Market participants are buyers and sellers in the principal market that are (i) independent, (ii) knowledgeable, (iii) able to transact, and (iv) willing to transact. The guidance prioritizes the inputs used in measuring fair value into the following hierarchy:

Level 1- Quoted prices (unadjusted) in active markets for identical assets or liabilities;

Level 2- Inputs other than quoted prices included within Level 1 that are either directly or indirectly observable; and

Level 3- Unobservable inputs in which little or no market activity exists, therefore requiring an entity to develop its own assumptions about the assumptions that market participants would use in pricing.

The following table summarizes the assets and liabilities measured at fair value on a recurring basis (in thousands):

| December 31, 2017               |           |           |          |         |
|---------------------------------|-----------|-----------|----------|---------|
|                                 | Total     | Level 1   | Level 2  | Level 3 |
| Assets                          |           |           |          |         |
| Cash and cash equivalents:      |           |           |          |         |
| Cash                            | \$1,026   | \$1,026   | \$—      | \$ —    |
| Money market funds              | 106,677   | 106,677   | —        | —       |
| Total cash and cash equivalents | 107,703   | 107,703   | —        | —       |
| Short-term investments:         |           |           |          |         |
| Corporate debt securities       | 24,241    | —         | 24,241   | —       |
| Commercial paper                | 18,893    | —         | 18,893   | —       |
| Total short-term investments    | 43,134    | —         | 43,134   | —       |
| Total                           | \$150,837 | \$107,703 | \$43,134 | \$ —    |
| December 31, 2016               |           |           |          |         |
|                                 | Total     | Level 1   | Level 2  | Level 3 |
| Assets                          |           |           |          |         |
| Cash and cash equivalents:      |           |           |          |         |
| Cash                            | \$2,728   | \$2,728   | \$—      | \$ —    |
| Money market funds              | 19,655    | 19,655    | —        | —       |
| Total cash and cash equivalents | 22,383    | 22,383    | —        | —       |
| Short-term investments:         |           |           |          |         |
| Corporate debt securities       | 20,619    | —         | 20,619   | —       |
| Commercial paper                | 13,732    | —         | 13,732   | —       |
| Total short-term investments    | 34,351    | —         | 34,351   | —       |



|       |          |          |          |    |   |
|-------|----------|----------|----------|----|---|
| Total | \$56,734 | \$22,383 | \$34,351 | \$ | — |
|-------|----------|----------|----------|----|---|

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The Company's investments in Level 1 assets are valued based on publicly available quoted market prices for identical securities as of December 31, 2017 and 2016. The Company determines the fair value of Level 2 related securities with the aid of valuations provided by third parties using proprietary valuation models and analytical tools. These valuation models and analytical tools use market pricing or prices for similar instruments that are both objective and publicly available, including matrix pricing or reported trades, benchmark yields, broker/dealer quotes, issuer spreads, two-sided markets, benchmark securities, bids and/or offers. There were no transfers between fair value measurement levels for the years ended December 31, 2017 and 2016.

#### 6. Other Current Assets and Other Long-Term Assets

Other current assets consisted of the following (in thousands):

|                                | December 31, |         |
|--------------------------------|--------------|---------|
|                                | 2017         | 2016    |
| Prepaid expenses               | \$3,085      | \$1,879 |
| Deposits and other receivables | 1,600        | 759     |
| Interest receivables           | 237          | 183     |
|                                | \$4,922      | \$2,821 |

The other long-term assets balance as of December 31, 2017 consists of \$1.0 million in deposits paid in conjunction with the Company's research and development activities compared to \$3.3 million as of December 31, 2016.

#### 7. Property and Equipment, Net

Property and equipment, net consisted of the following (in thousands):

|                                       | December 31, |       |
|---------------------------------------|--------------|-------|
|                                       | 2017         | 2016  |
| Computer equipment                    | \$329        | \$329 |
| Office and other equipment            | 301          | 301   |
| Laboratory equipment                  | 643          | 563   |
| Leasehold improvements                | 63           | 63    |
| Gross property and equipment          | 1,336        | 1,256 |
| Less: Accumulated depreciation (811 ) | (627 )       |       |
| Property and equipment, net           | \$525        | \$629 |

The Company incurred depreciation expense of \$0.2 million during the years ended December 31, 2017, 2016 and 2015, respectively.

#### 8. Accounts Payable and Accrued Liabilities

Accounts payable and accrued liabilities consisted of the following (in thousands):

|                                                  | December 31, |          |
|--------------------------------------------------|--------------|----------|
|                                                  | 2017         | 2016     |
| Accounts payable                                 | \$4,344      | \$6,296  |
| Accrued clinical, development and other expenses | 6,256        | 5,743    |
| Accrued compensation and benefits                | 3,021        | 2,923    |
| Other current liabilities                        | 23           | 40       |
|                                                  | \$13,644     | \$15,002 |



## 9. Stockholders' Equity

### Common Stock

The following shares were reserved for future issuance:

|                                                                 | December 31,<br>2017 |
|-----------------------------------------------------------------|----------------------|
| Common stock options outstanding and available for future grant | 6,306,836            |
| Warrants to purchase common stock                               | 11,396,262           |
| Employee Stock Purchase Plan                                    | 178,896              |
|                                                                 | 17,881,994           |

### Sale of Common Stock

In November 2017, the Company sold 2,938,986 shares of its common stock at a public offering price of \$13.00 per share and sold warrants to purchase up to 4,137,999 shares of its common stock at a public offering price of \$12.999 per warrant share. After deducting underwriter discounts and offering expenses, the Company received net proceeds from the transaction of \$86.7 million. In January 2017, the Company sold 5,002,702 shares of its common stock at a public offering price of \$5.60 per share and sold warrants to purchase up to 7,258,263 shares of its common stock at a public offering price of \$5.599 per warrant share. After deducting underwriter discounts and offering expenses, the Company received net proceeds from the transaction of \$66.8 million. In both cases, the public offering price for the warrants was equal to the public offering price of the common stock, less the \$0.001 per share exercise price of each warrant.

These warrants were recorded as a component of stockholders' equity within additional paid-in capital. Per their terms, the outstanding warrants to purchase shares of common stock may not be exercised if the holder's ownership of the Company's common stock would exceed 19.99 percent, for certain holders, and 9.99 percent for other holders, following such exercise.

### Warrants

As of December 31, 2017, the following warrants for common stock were issued and outstanding:

| Issue date        | Expiration date | Exercise price | Number of warrants outstanding |
|-------------------|-----------------|----------------|--------------------------------|
| January 11, 2017  | None            | \$ 0.001       | 7,258,263                      |
| November 20, 2017 | None            | \$ 0.001       | 4,137,999                      |
|                   |                 |                | 11,396,262                     |

During the year ended December 31, 2017, warrants for 52,825 shares of the Company's common stock were exercised via cashless exercises and 585,729 shares were exercised for cash generating proceeds of \$4.6 million resulting in the issuance of a total of 638,554 shares of common stock.

During the year ended December 31, 2016, warrants for 289,789 shares of the Company's common stock were exercised via cashless exercises and 313,756 shares were exercised for cash generating proceeds of \$2.1 million resulting in the issuance of a total of 603,545 shares of common stock.

During the year ended December 31, 2015, warrants for 1,037,330 shares of the Company's common stock were exercised via cashless exercises resulting in the issuance of a total of 809,498 shares of common stock.

## 10. Share-Based Compensation

## Equity Incentive Plan

The Company has in place a stock option plan (the "Stock Option Plan") for the benefit of employees, directors, officers and consultants of the Company. In May 2013 our Board of Directors adopted the 2013 Equity Incentive Plan (the "2013 Plan"). The 2013 Plan was approved by our stockholders in connection with the Arrangement. Our Board of Directors and stockholders approved an amendment to the 2013 Plan in 2017 to, among other things, increase the aggregate number of shares of common stock authorized for issuance under the 2013 Plan by 2.9 million shares. The 2013 Plan is a continuation of and successor to the Stock Option Plan and no further grants will be made under the Stock Option Plan. As of December 31, 2017, there were approximately 3.0 million stock options available to be issued.

To date, share-based compensation awards under either the Stock Option Plan or the 2013 Plan consist of incentive and non-qualified stock options. Stock options granted under each of the plans must have an exercise price equal to at least 100% of the fair market value of our common stock on the date of grant and generally vest over four years. The Stock Option Plan has contractual terms ranging from five to seven years and the 2013 Plan has contractual terms ranging from seven to ten years.

The following table summarizes our stock option activity and related information for the year ended December 31, 2017:

|                                                          | Number of options | Weighted average exercise price | Weighted-Average Remaining Contractual Term (years) | Aggregate Intrinsic Value (millions) |
|----------------------------------------------------------|-------------------|---------------------------------|-----------------------------------------------------|--------------------------------------|
| Balance outstanding as of December 31, 2016              | 2,813,342         | \$ 16.57                        |                                                     |                                      |
| Granted                                                  | 970,226           | \$ 5.36                         |                                                     |                                      |
| Exercised                                                | (45,573 )         | \$ 8.74                         |                                                     |                                      |
| Canceled/forfeited                                       | (415,908 )        | \$ 14.47                        |                                                     |                                      |
| Expired                                                  | (17,906 )         | \$ 12.50                        |                                                     |                                      |
| Balance outstanding as of December 31, 2017              | 3,304,181         | \$ 13.67                        | 7.2                                                 | \$ 21.0                              |
| Options exercisable at December 31, 2017                 | 1,843,808         | \$ 16.28                        | 6.1                                                 | \$ 7.3                               |
| Options vested and expected to vest at December 31, 2017 | 3,304,181         | \$ 13.67                        | 7.2                                                 | \$ 21.0                              |

The total intrinsic value of stock options exercised was \$0.3 million, \$0.3 million and \$0.6 million for the years ended December 31, 2017, 2016, and 2015, respectively. The Company received total cash of \$0.4 million, \$0.2 million and \$0.6 million for the exercise of options for the years ended December 31, 2017, 2016 and 2015, respectively. The total fair value of options vested during the years ended December 31, 2017, 2016 and 2015 was \$10.6 million, \$8.6 million and \$6.5 million, respectively. Upon option exercise, the Company issues new shares of its common stock.

Total share-based compensation expense by statement of operations classification is presented below (in thousands):

|                                  | Year ended December 31, |         |         |
|----------------------------------|-------------------------|---------|---------|
|                                  | 2017                    | 2016    | 2015    |
| Research and development expense | \$3,192                 | \$5,461 | \$3,669 |

|                                    |         |          |          |
|------------------------------------|---------|----------|----------|
| General and administrative expense | 3,594   | 5,163    | 6,585    |
|                                    | \$6,786 | \$10,624 | \$10,254 |

For the years ended December 31, 2017, 2016 and 2015, no share-based compensation expense was capitalized and there were no recognized tax benefits associated with the share-based compensation charge.

The fair value of options granted is estimated at the date of grant using the Black-Scholes option pricing model. The assumptions used for the specified reporting periods and the resulting estimates of weighted-average estimated fair value per share of options granted during those periods are as follows:

|                                                 | Year Ended December 31, |         |         |
|-------------------------------------------------|-------------------------|---------|---------|
|                                                 | 2017                    | 2016    | 2015    |
| Risk-free interest rate                         | 2.1%                    | 1.5%    | 1.5%    |
| Dividend yield                                  | —%                      | —%      | —%      |
| Volatility factor                               | 96.0%                   | 101.7%  | 104.3%  |
| Expected term (in years)                        | 6.0                     | 6.0     | 6.0     |
| Weighted average estimated fair value per share | \$4.17                  | \$13.32 | \$19.44 |

**Risk-Free Interest Rate** - The risk-free interest rate is the rate for periods equal to the expected term of the stock option based on U.S. Treasury zero-coupon bonds.

**Dividend Yield** - The dividend yield is based on the Company's history and expectation of dividend payouts. The Company has not paid, and does not intend to pay dividends.

**Volatility Factor** - The expected volatility assumption was determined by examining the historical volatility of the Company's stock.

**Expected Term** - The expected term represents the weighted average period the stock options are expected to be outstanding. The Company uses the simplified method for estimating the expected term as provided by the Securities and Exchange Commission. The simplified method calculates the expected term as the average time-to-vesting and the contractual life of the options. The Company believes this methodology is appropriate given the Company's limited history as a U.S. public company.

The total compensation cost not yet recognized as of December 31, 2017 related to non-vested option awards was \$4.4 million which will be recognized over a weighted-average period of 1.2 years.

## 2013 Employee Stock Purchase Plan

In May 2013, the Company's Board of Directors adopted the ESPP. The ESPP was approved by the Company's stockholders in connection with the Arrangement. In December 2014, the ESPP became effective and the first purchase period began. The ESPP permits eligible employees to make payroll deductions to purchase up to \$25,000 of the Company's common stock on regularly scheduled purchase dates at a discount. Offering periods under the ESPP are not more than six months in duration and shares are purchased at 85% of the lower of the closing price for the Company's common stock on the first day of the offering period or the date of purchase. The ESPP initially authorized the issuance of 300,000 shares of the Company's common stock pursuant to rights granted to employees for their payroll deductions. As of December 31, 2017, 121,104 shares have been issued out of the plan.

## 11. Employee Benefit Plan

The Company has a defined contribution 401(k) plan (the "Plan") for all employees. Employees are eligible to participate in the Plan if they are at least 21 years of age or older. Under the terms of the Plan, employees may make voluntary contributions as a percentage of compensation. The Company matches up to 4% of an employee's contributions, subject to a limit of \$2,500 per year. Expense associated with the Company's matching contribution totaled \$0.1 million for the years ended December 31, 2017, 2016 and 2015, respectively.





## 12. Income Taxes

The Company had no federal income tax expense and immaterial state tax expense for the years ended December 31, 2017, 2016 and 2015.

The differences between the effective income tax rate and the statutory tax rates during the years ended 2017, 2016 and 2015 are as follows (in thousands):

|                                                          | Year Ended December 31, |            |            |
|----------------------------------------------------------|-------------------------|------------|------------|
|                                                          | 2017                    | 2016       | 2015       |
| Net loss before tax                                      | \$(70,430)              | \$(83,118) | \$(64,544) |
| Statutory combined U.S. federal and state tax rate       | 34.00 %                 | 34.00 %    | 34.00 %    |
| Statutory federal and state taxes                        | (23,946 )               | (28,260 )  | (21,945 )  |
| Increase (decrease) in taxes recoverable resulting from: |                         |            |            |
| Effect of change in valuation allowance                  | (4,154 )                | 28,446     | 22,350     |
| Non-deductible share-based compensation                  | 695                     | 1,247      | 923        |
| Tax credits                                              | (2,563 )                | (2,906 )   | (2,430 )   |
| Share issue costs - temporary difference                 | —                       | (78 )      | (184 )     |
| Write off of Methylgene US NOL                           | 307                     | —          | —          |
| Differential in income tax rates of foreign subsidiary   | (169 )                  | 261        | 31         |
| Change in tax rate                                       | 303                     | —          | —          |
| Tax Cuts and Jobs Act                                    | 28,569                  | —          | —          |
| Uncertain tax positions                                  | 646                     | 3,921      | 1,961      |
| Return to provision and other true-ups                   | 368                     | (2,619 )   | (899 )     |
| Other differences                                        | (56 )                   | (12 )      | 193        |
| Income tax benefit                                       | \$—                     | \$—        | \$—        |

## Deferred Tax

The following table summarizes the significant components of our deferred tax assets (in thousands):

|                                                               | December 31, |           |
|---------------------------------------------------------------|--------------|-----------|
|                                                               | 2017         | 2016      |
| Deferred tax assets:                                          |              |           |
| Tangible and intangible depreciable assets                    | \$17,927     | \$ 990    |
| Stock compensation                                            | 4,976        | 6,635     |
| Provisions                                                    | 611          | 853       |
| Net operating loss carry forwards                             | 44,598       | 66,489    |
| Capital loss carryforward                                     | 83           | 51        |
| Scientific research and experimental development expenditures | 5,467        | 5,531     |
| Research and development tax credits                          | 7,016        | 4,266     |
| Total gross deferred tax assets                               | 80,678       | 84,815    |
| Less valuation allowance                                      | (80,678 )    | (84,815 ) |
| Net deferred tax assets                                       | \$—          | \$ —      |

Total valuation allowance decreased by \$4.1 million for the year ended December 31, 2017. The Company has established a full valuation allowance against its deferred tax assets as of December 31, 2017 due to the uncertainty surrounding the realization of such assets as evidenced by the cumulative losses from operations through December 31, 2017.

For Canadian federal income tax purposes, the Company's Canadian federal scientific research and experimental development expenditures amounted to \$19.9 million at December 31, 2017, 2016 and 2015 and for provincial income tax purposes amounted to \$21.6 million at December 31, 2017, 2016 and 2015. As operations in Canada ceased during 2014, no expenditures

were incurred for the years ended December 31, 2017, 2016 and 2015. These expenditures are available to reduce future taxable income and have an unlimited carry forward period. Scientific research and development expenditures are subject to verification by the taxation authorities, and accordingly, these amounts may vary by a material amount. In addition, the Company has research and development tax credit carryforwards for U.S. federal and state income tax purposes as of December 31, 2017 of \$7.2 million and \$2.9 million, respectively. The federal credits will begin to expire in 2033 unless utilized and the state credits have an indefinite life.

At December 31, 2017, the Company's net operating loss carry forwards ("NOLs") for U.S. federal and state income taxes were \$110.3 million and \$77.3 million, respectively and the Company's NOLs for Canadian federal and provincial income tax purposes were \$80.6 million and \$79.9 million, respectively. The NOLs are available to offset future taxable income from both U.S. federal and state tax sources, as well as Canadian federal and provincial tax sources and the tax benefits of which have not been recognized in the consolidated financial statements. The NOLs expire as follows (in thousands):

|             | US        |          | Canada   |            |
|-------------|-----------|----------|----------|------------|
|             | Federal   | State    | Federal  | Provincial |
| Expires in: |           |          |          |            |
| 2030        | \$—       | \$—      | \$5,907  | \$ 5,985   |
| 2031        | —         | —        | 7,059    | 7,066      |
| 2032        | —         | —        | 13,308   | 12,433     |
| 2033        | 2,225     | 2,232    | 18,623   | 19,385     |
| 2034        | 7,276     | 22,162   | 32,401   | 31,809     |
| 2035        | 53,359    | 52,950   | 1,084    | 1,084      |
| 2036        | 23,379    | —        | 777      | 777        |
| 2037        | 24,044    | —        | 1,408    | 1,408      |
|             | \$110,283 | \$77,344 | \$80,567 | \$ 79,947  |

The future utilization of the U.S. federal and state NOL carryforwards to offset future taxable income may be subject to an annual limitation as a result of ownership changes that may have occurred previously or may occur in the future. The Tax Reform Act of 1986 (the "1986 Act") limits a company's ability to utilize certain tax credit carryforwards and net operating loss carryforwards in the event of a cumulative change in ownership in excess of 50% as defined in the Act. During 2017, the Company completed a study to assess whether an ownership change, as identified by Section 382 of the 1986 Act, had occurred from the Company's formation through December 31, 2017. Based upon the study, the Company determined an ownership change had occurred during 2017, causing the annual utilization of the NOL and credit carryforwards to be limited. The Company does not believe any of the NOL and credit carryforwards generated through December 31, 2017 would expire solely as a result of annual limitations on the utilization of those attributes. The Canadian Federal and Provincial Tax Acts maintain similar rules in the case of acquisition of control, which may limit the utilization of tax attributes.

The Company files income tax returns in the U.S. (federal and state) and Canada (federal and provincial). The Company's U.S. operations have not been audited for any open taxation years. The Company has experienced losses for U.S. tax purposes and therefore, the taxation authorities may review any loss year, if and when the losses are utilized.

For Canadian tax purposes, the Company remains subject to federal and provincial audit for the December 31, 2013 and subsequent taxable years. Where taxation years remain open, the Company considers it reasonably possible that issues may be raised or tax positions agreed to with the taxation authorities, which may result in increases or decreases of the balance of non-refundable ITCs and NOLs. However, an estimate of such increases and decreases cannot be currently made.



A reconciliation of the beginning and ending amounts of unrecognized tax positions are as follows (in thousands):

|                                               | Federal<br>December 31, |         |       | Provincial/State<br>December 31, |         |         |
|-----------------------------------------------|-------------------------|---------|-------|----------------------------------|---------|---------|
|                                               | 2017                    | 2016    | 2015  | 2017                             | 2016    | 2015    |
| Unrecognized tax positions, beginning of year | \$1,095                 | \$509   | \$42  | \$7,333                          | \$2,274 | \$18    |
| Gross increase — current period tax positions | 588                     | 598     | 445   | 227                              | 195     | 259     |
| Gross decrease — prior period tax positions   | —                       | (9)     | (4)   | (3)                              | —       | (3)     |
| Gross increase — prior period tax positions   | 11                      | —       | 26    | —                                | 4,866   | 2,000   |
| Expiration of statute of limitations          | (1)                     | (3)     | —     | (1)                              | (2)     | —       |
| Unrecognized tax positions, end of year       | \$1,693                 | \$1,095 | \$509 | \$7,556                          | \$7,333 | \$2,274 |

If recognized, none of the unrecognized tax positions would impact the Company's income tax benefit or effective tax rate as long as the Company's deferred tax assets remain subject to a full valuation allowance. The Company does not expect any significant increases or decreases to the Company's unrecognized tax positions within the next 12 months.

The Company recognizes interest and penalties related to unrecognized tax benefits in income tax expense. The Company had no accrual for interest or penalties on tax matters as of December 31, 2017 and 2016 and the Company had no ongoing tax audits as of December 31, 2017.

On December 22, 2017, the President of the United States signed into law the Tax Cuts and Jobs Act (the "2017 Act"). The 2017 Act amends the Internal Revenue Code to reduce tax rates and modify policies, credits, and deductions for individuals and businesses. For businesses, the Act reduces the corporate tax rate from a maximum of 35% to a flat 21% rate. The rate reduction is effective on January 1, 2018. The provisional amount related to the re-measurement of certain deferred tax assets, based on the rates at which they are expected to reverse in the future, was \$28.6 million. Due to the Company's full valuation allowance position, there was no net impact on the Company's income tax provision for the year ended December 31, 2017 as the reduction in the deferred tax asset balance was fully offset by a corresponding decrease in the valuation allowance.

In conjunction with the 2017 Act, the SEC staff issued Staff Accounting Bulletin No. 118 to address the application of U.S. GAAP in situations when a registrant does not have the necessary information available, prepared, or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of the 2017 Act. The Company has recognized the provisional tax impacts related to deemed repatriated earnings and the revaluation of deferred tax assets and liabilities at December 31, 2017. There was no net impact on the Company's consolidated financial statements for the year ended December 31, 2017 as the corresponding adjustment was made to the valuation allowance. The ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, additional analysis, changes in interpretations and assumptions the Company has made, additional regulatory guidance that may be issued, and actions the Company may take as a result of the 2017 Act.

### 13. Investment Tax Credits

In prior years, the Company was entitled to claim Canadian federal and provincial ITCs for eligible scientific research and development expenditures. The Company recorded ITCs based on management's best estimates of the amount to be recovered and ITCs claimed are subject to audit by the taxation authorities and accordingly, may vary by a material amount. The Company has not recorded federal or provincial ITCs since the year ended December 31, 2013, as the primary operations of the Company were moved from Canada to San Diego, California in early 2014.

The Company's non-refundable Canadian federal ITCs as of December 31, 2017 are \$3.9 million and relate to scientific research and development expenditures, which may be utilized to reduce Canadian federal income taxes payable in future years. The benefits of the non-refundable Canadian federal ITCs have not been recognized in the financial statements and will be recorded as a reduction of tax expense when realized.

The non-refundable investment tax credits expire as follows (in thousands):

|             | Federal<br>ITC |
|-------------|----------------|
| Expires in: |                |
| 2030        | \$764          |
| 2031        | 1,000          |
| 2032        | 1,125          |
| 2033        | 1,018          |
|             | \$3,907        |

#### 14. Commitments and Contingencies

On June 24, 2014, the Company entered into a lease agreement for approximately 18,000 square feet of completed office and laboratory space located in San Diego, California. The office space under the lease is the Company's corporate headquarters. The lease commenced in two phases (in July 2014 and March 2015) at a combined total initial monthly rent of \$24,100 per month. The leased property is subject to a 3% annual rent increase following availability that results in the Company recording deferred rent over the term of the lease. In addition to such base monthly rent, the Company is obligated to pay certain customary amounts for its share of operating expenses and facility amenities. The original lease provided for expiration on January 31, 2018. On March 23, 2017, the Company entered into a First Amendment to Lease Agreement to amend the original lease agreement and to extend the term of the original lease for one year through January 31, 2019. All other terms and covenants from the original lease agreement remain unchanged.

Future minimum payments required under the lease are summarized as follows (in thousands):

| Year Ending December 31:     |       |
|------------------------------|-------|
| 2018                         | \$289 |
| 2019                         | 26    |
| Thereafter                   | —     |
| Total minimum lease payments | \$315 |

Total lease expense for the years ended December 31, 2017, 2016 and 2015 was \$0.7 million, \$0.8 million, and \$0.6 million, respectively.

#### 15. Selected Quarterly Financial Data (Unaudited)

The following is a summary of the quarterly results of the Company for the years ended December 31, 2017 and 2016 (unaudited, in thousands, except for per share data):

|                | Three Months Ended |            |            |            | Year Ended<br>December 31,<br>2017 |
|----------------|--------------------|------------|------------|------------|------------------------------------|
|                | 3/31/17            | 6/30/17    | 9/30/17    | 12/31/17   |                                    |
| Operating loss | \$(18,090)         | \$(18,616) | \$(16,601) | \$(18,228) | \$(71,535)                         |
| Net loss       |                    |            |            |            |                                    |