

Mirati Therapeutics, Inc.  
Form 10-K  
March 11, 2015  
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, 2014; 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)

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

9363 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: None

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

Common Stock, \$0.001 par value per share

(Title of Class)

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Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ 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 ☐

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" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer <input type="checkbox"/>	Accelerated filer <input checked="" type="checkbox"/>
Non-accelerated filer <input type="checkbox"/>	Smaller reporting company <input type="checkbox"/>

(Do not check if a smaller reporting company)

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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 \$174.9 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 6, 2015, the registrant had 16,164,311 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 2015 Annual Meeting of Stockholders, which will be held on May 21, 2015 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, or this 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 future 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 future product candidates;
- the size and growth potential of the markets for our future product candidates, and our ability to serve those markets;
- our ability to successfully commercialize our future product candidates;
- the rate and degree of market acceptance of our future 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, or the JOBS Act;

- the loss of key scientific or management personnel; and
- our other future financial results, capital requirements and need for additional financing.

Item 1. Business

**BUSINESS**

**Overview**

We are a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. We focus our development programs on drugs intended to treat specific genetically defined and selected cancer patients with unmet needs. Our pipeline consists of three product candidates: MGCD265, MGCD516 and mocetinostat. MGCD265 and MGCD516 are orally-bioavailable, spectrum-selective kinase inhibitors with distinct target profiles. Both MGCD265 and MGCD516 are in development to treat patients with non-small cell lung cancer, or NSCLC, and other solid tumors. MGCD265 is in Phase 1b clinical development and MGCD516 is in the dose escalation phase of Phase 1 clinical development. Mocetinostat is an orally-bioavailable, spectrum-selective histone deacetylase, or HDAC, inhibitor currently in Phase 2 development. Mocetinostat is being developed for the second line treatment of patients with bladder cancer and non-hodgkins lymphoma, or NHL, specifically focusing on diffuse large B-cell lymphoma, or DLBCL, and follicular lymphoma, or FL. Selected bladder cancer, DLBCL and FL tumors have loss of function and genetic alterations in genes that have been shown to increase their sensitivity of their tumor cells to mocetinostat in preclinical models.

We believe that an increased understanding of the genomic factors that drive tumor cell growth can lead to the development of cancer drugs that target these genomic factors, resulting in increased efficacy while reducing side effects. We are leveraging this knowledge to develop targeted cancer therapies to address unmet needs in selected cancer patient populations. Our novel kinase inhibitors are intended to target specific mutations that drive the growth of cancer or are implicated in cancer drug resistance or pathogenic processes such as tumor angiogenesis. Our HDAC inhibitor, mocetinostat, acts through important epigenetic mechanisms that are dysregulated in certain cancers. We plan to identify additional opportunities by leveraging our deep scientific understanding of molecular drug targets and mechanisms of resistance and potentially in-licensing or internally discovering promising, early-stage novel drug candidates.

Our three clinical stage product candidates are as follows:

MGCD265 is an orally-bioavailable, potent, small molecule kinase inhibitor of MET and Axl receptor tyrosine kinases, or RTKs. MGCD265 is in development for the treatment of solid tumors, with an initial focus on NSCLC but including other solid tumors including gastroesophageal cancers and squamous cell carcinoma of the head and neck, or HNSCC. In 2014 we completed development of a new formulation to improve plasma exposure thereby improving the degree of target inhibition to levels which we believe can be sufficient to demonstrate single agent clinical activity in patients with genetic alterations of MET and Axl. In late 2014 we established the maximum tolerated dose, or MTD, for the new formulation and initiated dose expansion cohorts in patients selected for certain genetic driver mutations that activate the MET and Axl pathways. The patient selection strategy based upon these genetic mutations is designed to result in a high response rate that could enable an accelerated development pathway. We anticipate initial data regarding clinical proof of concept by mid-2015 and, if positive, to begin a single-arm registration trial in the second half of 2015.

MGCD516 is an orally-bioavailable, potent, small molecule spectrum-selective kinase inhibitor in development for the treatment of solid tumors with an emphasis on genetic alterations involving the Trk, RET and DDR RTK families. We plan to focus on solid tumors exhibiting genetic alterations or dysregulation of these key drivers of tumor growth, initially in NSCLC. In addition, we plan to evaluate other tumor types where the profile of MGCD516 would suggest clinical benefit. An ongoing Phase 1 dose escalation study is designed to identify the optimal biologic dose or MTD and evaluate a cohort of patients selected for key driver mutations in Trk, RET and DDR receptor families. Based upon preclinical and early clinical information, we believe that we will reach a dose that potentially inhibits the targeted genetic alterations in the first half of 2015 and initiate dose expansion cohorts in selected patients in mid-2015. We believe that initial data on clinical activity in patients with genetic alterations of Trk, RET or DDR family members could be available in the second half of 2015.

Mocetinostat is an orally-bioavailable, spectrum-selective HDAC inhibitor in Phase 2 clinical trials in patients with bladder cancer, myelodysplastic syndrome, or MDS, and NHL, specifically DLBCL and FL. Patients in the bladder cancer and NHL Phase 2 studies are selected for tumors with genetic alteration in two histone acetyl transferase genes, or HATs, that regulate histone acetylation and that have been shown to increase the sensitivity of tumor cells to mocetinostat in preclinical models. We are also evaluating mocetinostat for the first line treatment of patients with MDS in combination with Vidaza, a hypomethylating agent, or HMA. We believe that this is the first and only ongoing clinical development plan for an HDAC inhibitor in a genetically selected subset of patients. We have completed 13 clinical trials with mocetinostat which enrolled approximately 450 patients with a variety of hematologic malignancies and solid tumors.

We anticipate initial proof of concept clinical data in bladder cancer and DLBCL by mid-2015, which, if positive, could enable the initiation of single agent registration trials.

We were incorporated under the laws of the State of Delaware on April 29, 2013 as Mirati Therapeutics, Inc. On May 8, 2013, we entered into a plan of arrangement with MethylGene, Inc., or MethylGene Canada, pursuant to which MethylGene Canada became our wholly owned subsidiary and all of its shareholders became proportionate shareholders of ours. 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 of 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").

## Our Strategy

Our goal is to be a leading developer of targeted cancer therapies for genetically selected patient populations. The key components of our strategy include:

Develop a pipeline of targeted cancer therapies. We believe that an increased understanding of the genomic factors that drive tumor cell growth will lead to the development of cancer drugs with increased efficacy while reducing side effects. We are leveraging the prior successful experience of certain members of our management team in the development and approval of targeted oncology drugs (crizotinib or Xalkori) to develop targeted cancer therapies to address unmet needs in specific cancer populations. Our clinical pipeline is comprised of two novel kinase inhibitors that target specific mutations that drive cancer cell growth and an HDAC inhibitor which is one of the most advanced epigenetic therapies in development. We plan to identify additional targets by leveraging our deep scientific understanding of molecular drug targets and mechanisms of resistance through internal drug discovery activities or potentially in-licensing promising, early-stage novel drug candidates.

Employ efficient and flexible approaches to accelerate clinical development. We will pursue indications and select specific patient populations in which activity of our product candidates can be assessed in small proof of concept, or POC, clinical trials leading to accelerated clinical development. When designing clinical trials, we structure our clinical development approach to test multiple clinical hypotheses in a single trial and design trials with the flexibility to adapt quickly and accelerate once a signal of clinical benefit is observed. We believe our approach may increase the likelihood of seeing results early in clinical trials with fewer patients, reducing our clinical development risk and development costs and allowing us to potentially accelerate the development of our product pipeline.

Advance our two lead kinase inhibitors. Kinase inhibitors have significantly improved the care of many cancer patients and represent a commercially successful category of targeted cancer therapies with global sales of over \$29.1 billion in 2011, according to BCC Research. We have two internally discovered novel kinase inhibitors in development: MGCD265 and MGCD516. These product candidates target pathways of high scientific interest, including MET, Axl, Trk, RET, and DDR RTK families and are believed to be drivers of tumor growth and responsible for the development of tumor resistance to several anti-cancer treatments. MGCD265 is in Phase 1b development and MGCD516 is in Phase 1 development in the dose escalation portion of the trial. In the second half of 2015, we plan to initiate a registration trial for MGCD265 following POC and initiate dose expansion cohorts for MGCD516 in selected patients once we achieve a dose at which we are confident our targets are sufficiently inhibited.

- Advance mocetinostat, our HDAC inhibitor. HDAC inhibitors have been shown to be effective in treating hematologic malignancies, as evidenced by the approvals of Istodax and Zolinza. We have completed 13



clinical trials with mocetinostat in approximately 450 patients. We are focused on the development of mocetinostat in the treatment of bladder cancer and NHL, specifically DLBCL and FL, in patients whose tumors have certain genetic alterations in one of two genes that regulate histone acetylation, CREBBP and EP300. Certain alterations in the CREBBP and EP300 genes have been shown to increase the sensitivity of tumor cells or cancer models to mocetinostat in preclinical studies. We are also evaluating mocetinostat for the first line treatment of patients with MDS in combination with Vidaza, an HMA. Phase 2 trials of single agent mocetinostat in bladder cancer and NHL (DLBCL and FL) are ongoing. We anticipate initial proof of concept clinical data in bladder cancer patients and DLBCL patients by the mid-2015.

Leverage partnerships to develop our product candidates. We plan to collaborate with third parties and partner certain rights to our product candidates as a means to accelerate their broader clinical development and maximize their therapeutic

and market potential. We plan to retain certain key development and commercialization rights in our partnerships. We believe that retaining this strategic flexibility will enable us to maximize shareholder value.

#### Product Candidates

The following chart depicts the current state of our oncology development programs:

PRODUCT CANDIDATE	INDICATION	TARGETS	COMMERCIAL RIGHTS	STAGE OF DEVELOPMENT AND ANTICIPATED MILESTONES
MGCD265	Solid Tumors	MET, Axl	Mirati: Global	Initial data from Phase 1b expansion cohorts in selected patients in mid-2015. Initiate Registration Trial in second half of 2015.
MGCD516	Solid Tumors	Trk, RET,DDR	Mirati: Global	Phase 1 dose escalation ongoing. Initiate expansion cohorts in the second half of 2015.
Mocetinostat	Bladder Cancer and NHL (DLBCL and FL)	HDACs 1, 2, 3, 11	Taiho: Certain Asian Territories Mirati: All Other Territories	Phase 2 in bladder cancer ongoing. Phase 2 in DLBCL and FL ongoing. Initial POC data in bladder in mid-2015. Initial POC data in DLBCL in mid-2015.

#### Our Targeted Kinase Programs

Targeted therapies selectively inhibit specific genes or pathways that are inappropriately activated in certain types of cancer cells and not in normal tissue, called driver mutations. RTKs are a family of kinases involved in the transmission of signals that regulate intercellular processes, including those that control cell growth and cell division. RTKs may be inappropriately activated in cancerous tissues resulting in uncontrolled tumor cell growth. Aberrant kinase function, caused by genetic mutations, gene amplification, or over-expression, underlies many cancer cell processes, making the kinome an important source for therapeutic targets in oncology. Discoveries of specific drivers of disease have led to the development of targeted therapies, or the tailoring of therapies to a particular tumor or disease profile. In some cases, these therapies have proven to be more efficacious while having fewer side effects than traditional non-targeted therapies, such as chemotherapy, which kill healthy cells along with cancer cells. Examples of successful development of oral targeted kinase inhibitors include Novartis AG's Gleevec, a BCR-ABL kinase inhibitor for the treatment of Philadelphia chromosome positive chronic myelogenous leukemia, and GlaxoSmithKline's Tykerb, a HER2 kinase inhibitor for the treatment of a subset of breast cancer patients over-expressing the HER2 kinase. Further examples of oral targeted kinase inhibitors include Pfizer's Xalkori and Bosulif and Bristol-Myers Squibb's Sprycel. We believe that therapies that target specific genetic abnormalities in subsets of cancer patients identified through diagnostic tests will result in streamlined clinical trials and improved patient outcomes and will be increasingly important in the continued evolution of the treatment of cancer.

We believe that by selecting patients whose tumors have genetic mutations and alterations in the pathways that are critical for tumor growth and are potently inhibited by our drugs, we will increase the potential for clinical benefit. A greater clinical benefit in selected patients would increase the likelihood of demonstrating clinical benefit earlier in development, potentially in Phase 1, which may allow us to move rapidly into registration trials. As a part of our ongoing development activities, we are using commercial diagnostic assays as well as assays developed internally for early clinical trials. We are working with external diagnostic providers to develop validated companion diagnostics for later stage clinical use and registration to ensure that the diagnostic is widely available for commercial use upon approval.

The clinical and commercial success of leading small molecule kinase inhibitors demonstrates the potential of new targeted treatments for cancer. BCC Research data indicates that the global kinase inhibitor market was \$29.1 billion in 2011, and is expected to reach \$40.2 billion by 2016. The following table lists retail sales figures for selected small molecule kinase inhibitors.

## 2013 Worldwide Retail Sales Figures of Selected Small Molecule Kinase Inhibitors

Brand Name	2013 Worldwide Sales(1) (in millions)
Gleevec	\$ 4,693
Tarceva	\$ 1,445
Sutent	\$ 1,204
Nexavar	\$ 1,024
Sprycel	\$ 1,280
Tykerb	\$ 324
Zelboraf	\$ 382
Xalkori	\$ 282

(1) Source: Evaluate Pharma.

Our kinase inhibitor programs in clinical development, MGCD265 and MGCD516, are kinase inhibitors with distinct target profiles. These new molecular entities are in development for the treatment of patients with NSCLC and other solid tumors that exhibit the mutations and alterations of interest. MGCD265 and MGCD516 were developed internally and we own all global rights to MGCD265 and MGCD516.

MGCD265 - A Multi-targeted Kinase Inhibitor for Solid Tumors

#### MGCD265 Overview

MGCD265 is an orally-bioavailable, potent, small molecule kinase inhibitor of MET and Axl. MGCD265 is in development for the treatment of patients with solid tumors. Our initial focus is on patients with NSCLC but we are investigating patients with other solid tumors including gastroesophageal cancers and HNSCC. In 2014 we completed development of a new formulation to improve plasma exposure. The new formulation is intended to maximize the degree of MET and Axl target inhibition and to increase the likelihood of seeing single agent clinical activity in selected patients. In late 2014 we established the maximum tolerated dose, or MTD, and initiated dose expansion cohorts in patients selected for specific driver mutations which activate the MET or Axl pathways. This patient selection strategy is designed to result in a high response rate that could enable an accelerated development pathway. We anticipate initial data regarding proof of concept in mid-2015 and, if positive, begin a registration trial in the second half of 2015.

Our development strategy for MGCD265 is based on our understanding of the compound's target inhibition profile and, accordingly, our initial focus for this program will be NSCLC although we intend to also explore other solid tumors such as gastroesophageal and HNSCC tumors where genetic alterations in MET or Axl are also known to be present. We intend to undertake patient selection using a targeted next generation sequencing assay to identify patients with certain genetic mutations or alterations of MET or Axl that result in oncogenic activation and are implicated as drivers of tumor progression.

#### MGCD265 Market Overview

The National Cancer Institute, or NCI, estimates that in 2014, approximately 224,210 patients in the United States were diagnosed with lung cancer and 159,260 died due to the disease. Approximately 85% of lung cancers are NSCLCs. The potential oncogenic mutations of MET and Axl that we are targeting may exist in up to 8% of NSCLC cases. At present, the prevalence of the genetic alterations of MET and Axl is less well characterized in other solid tumors, however, they are known to occur in other solid tumors and we are exploring those additional indications. Although other tumor types may respond to treatment with MGCD265, NSCLC, HNSCC and gastroesophageal

cancers are of particular relevance to demonstrate the clinical activity of MGCD265. Key features of these markets are shown in the table below.

## Estimated Market Size of Certain Cancer Therapies

Indication	Supporting Rationale	U.S. Annual Patient Incidence (United States, Europe and Japan)
Lung Cancer	Genetic alterations of MET and Axl in up to 8% of NSCLC	224,210
Head & Neck Cancer	Genetic alterations of MET and Axl in up to 8% of patients	42,440
Gastric Cancer	Genetic alterations of MET in up to 6% of patients	22,220

(1)Source: National Cancer Institute

Approximately 15% of NSCLC cases have activating EGFR mutations, equating to 28,650 patients each year in the United States. Although tyrosine kinase inhibitors that target EGFR have demonstrated efficacy in treating patients with EGFR mutations, tumors eventually become resistant to therapy. Resistance to EGFR therapy is mediated through mutation and/or overexpression of alternative targets and pathways, including MET and Axl in approximately 70% of resistant tumors, or 20,055 patients annually in the United States.

## MGCD265 Background

MGCD265 is a small molecule, spectrum-selective kinase inhibitor that potently inhibits MET and Axl. These targets have been shown to play key roles in tumor development, tumor cell survival, therapeutic resistance and blood vessel formation, or angiogenesis. MGCD265 is selective for these two targets at clinically achievable dose levels and shows minimal activity against a panel of over 300 other kinases. We believe this profile provides the following potential advantages for MGCD265:

- therapeutic action against specific mutations and genetic alterations of MET;
- therapeutic action against a novel target (Axl);
- high specificity reduces the risk of side effects from off-target activity; and
- the selection of patients whose tumors exhibit genetic alterations of MET or Axl that may be drivers of tumor growth provides an opportunity to demonstrate single agent clinical responses of MGCD265.

The MET receptor is a member of the RTK protein family that is found on the cell's surface that, when not properly regulated, plays a key role in the growth, survival and metastasis of various types of cancers. The MET target has generated significant scientific and pharmaceutical interest because of its direct involvement in tumor cell survival and angiogenesis. MET expression is elevated in several major tumor types including NSCLC, gastric cancer, RCC and HCC and is associated with poor prognosis. MET activation may also be associated with resistance to EGFR inhibitors such as Tarceva, Iressa and Erbitux. In tumors with EGFR mutation or activation, the activation or genetic alteration of MET is implicated as an escape mechanism leading to EGFR-inhibitor resistance. Inhibition of MET may result in clinical benefit by blocking the MET-driven escape mechanism used by some tumor cells when treated with other targeted inhibitors of the EGFR, such as Tarceva or Iressa.

Axl is also an RTK, and its expression has been shown to correlate with clinical-stage and lymph node status in NSCLC. Axl can be dysregulated in certain cancers through increased protein expression or gene rearrangement, resulting in abnormal tumor growth and tumor cell survival. Axl has also been linked to resistance to EGFR inhibitors such as Tarceva and Erbitux. Axl is also expressed in other tumor types and may be a clinically significant driver in RCC, ovarian, pancreatic and other tumors.

MGCD265 is distinguished from many other small molecule inhibitors of MET due to its potent activity against Axl which provides an opportunity against tumors driven by Axl such as NSCLC tumors that exhibit a translocation of Axl that drives tumor growth, thereby increasing the likelihood that these tumors will respond to MGCD265. Further, MET and Axl are both overexpressed and/or genetically altered in tumors that are resistant to EGFR inhibitors such as Tarceva, Iressa and Erbitux. It is estimated that MET is overexpressed in approximately half of EGFR-resistant tumors, and amplified in 5-20% of EGFR-resistant tumors. It is estimated that Axl is overexpressed in approximately 20-30% of EGFR-resistant tumors. The simultaneous inhibition of both MET and Axl pathways may be required for clinical efficacy in patients developing resistance to EGFR inhibitors or for the prevention of resistance by combining MGCD265 with an EGFR inhibitor as first line treatment. Finally, in preclinical studies MGCD265 has demonstrated inhibition of tumor cells which express mutant forms of MET that appears to be greater than other known small molecule inhibitors of MET.

The profile of MGCD265 and our clinical development strategy is clearly distinguished from MET antibody antagonists (such as MetMab) that inhibit MET pathway signaling primarily by preventing the binding of HGF to MET. The inhibition of the catalytic activity of MET via small molecule strategies like MGCD265 as opposed to inhibition of ligand binding by MET antibody antagonists is an important differentiated strategy in disease settings in which MET is activated by ligand-independent mechanisms including activating mutations, gene amplification, and/or extreme overexpression. Our primary focus in clinical development is on patients with NSCLC or other solid tumors exhibiting driver mutations in the MET and Axl pathways. These driver mutations result in constitutive activation of the MET or Axl receptors so they become independent of normally tightly regulated growth factor signaling. In the case of MET, genetic alterations can result in the activation of MET-dependent signaling independently of binding to HGF. Therefore, patients with these driver mutations would not be responsive to MET antibody antagonists that inhibit HGF binding but are more likely to respond to MGCD265, which inhibits signaling irrespective of growth factor binding. If we are able to demonstrate single agent POC in select patients, we also plan to explore the combination of MGCD265 with EGFR inhibitors to treat and/or prevent EGFR resistance.

#### MGCD265 Preclinical Development

Our preclinical studies, in a variety of in vivo tumor models, have suggested that MGCD265 is well tolerated at dose levels that inhibit MET and Axl and MGCD265 demonstrated tumor regression in experimental cancer models that exhibit genetic mutations and alterations of interest.

#### MGCD265 Clinical Trials

Multiple Phase 1 clinical trials have been conducted with MGCD265 showing evidence of clinical activity as monotherapy as well as in combination studies. While MGCD265 demonstrated antitumor activity as well as MET and Axl inhibition, it did not reach optimal plasma concentrations predicted to robustly inhibit MET. We have developed new formulations of MGCD265 that have demonstrated increased plasma exposure and in the second half of 2014 we reached the MTD with the new formulation and achieved exposures to reach greater than 90% inhibition of MET mutations, MET amplifications and Axl fusions. In the fourth quarter of 2014 we initiated expansion cohorts which are enrolling patients selected for specific genetic mutations and alterations of MET and Axl with initial proof of concept data for expansion cohorts anticipated in mid-2015.

The original IND for MGCD265 was filed in December 2007 and became effective in January 2008. Three schedules of continuous dosing of MGCD265 were evaluated sequentially in the ongoing monotherapy and combination studies: once daily (QD), twice daily (BID) and three times daily (TID). MGCD265 has been generally well tolerated at all doses and schedules tested to date, both as monotherapy and in combination with either Taxotere or Tarceva.



To date, 260 patients have been exposed to MGCD265 in multiple clinical trials in a variety of solid tumor types. To date, the most frequent treatment-related adverse events observed were diarrhea, fatigue and nausea. Other than as noted below, all of these trials were conducted with prior formulations of MGCD265 that are no longer actively being developed. In addition, none of those prior trials were conducted in patient populations that were selected for genetic alterations or mutations in MET and Axl that we expect are the most likely to respond to treatment with MGCD265, which is our current development focus.

The historical MGCD265 clinical trials are set forth in the following table.

## CLINICAL TRIALS EVALUATING MGCD265

Phase 1 Clinical Trial	Single Agent Dose Escalation, 21 day cycle	Completed (trial amended and continuing as described under Phase 1b clinical trial below)
Phase 1b Clinical Trial*	Single Agent Expansion Cohort in patients with genetic alterations of MET and Axl in NSCLC, HNSCC and other solid tumors, 21 day cycle	Ongoing
Phase 1/2 Clinical Trial	Combination with Erlotinib or Docetaxel in Subjects with advanced NSCLC, 21 day cycle	Completed

\*trial being conducted with new formulation

### Phase 1b Clinical Trial Evaluating MGCD265 in Solid Tumors (Ongoing)

MGCD265 is currently in an ongoing Phase 1b clinical study. In the second half of 2014 we established the MTD of a new formulation of MGCD265. The observed dose limiting toxicities, or DLTs, included one patient who experienced grade 3 fatigue and one patient that experienced grade 3 diarrhea. Clinical pharmacokinetic and pharmacodynamic data and nonclinical projections indicate MGCD265 plasma levels consistent with MET and Axl inhibition that we would expect to result in clinical activity. In the fourth quarter of 2014 we initiated the dose expansion portion of the trial and began enrolling patients selected for target alterations of interest in MET or Axl. The trial is ongoing and no data is yet available, however we anticipate initial proof of concept data by mid-2015.

### MGCD265 Developmental Initiatives and Objectives

Since January 2013, we have developed new formulations of MGCD265 designed to increase plasma exposure, improve the degree of target inhibition and increase the likelihood of seeing single agent clinical activity. We selected one of the new formulations and reached MTD in the second half of 2014. We believe the selected formulation and dose will be sufficient to achieve exposures to inhibit MET and Axl to a sufficient degree. In the fourth quarter of 2014 we initiated dose expansion cohorts in patients selected for mutations and alterations of MET or Axl that are implicated as drivers of tumor growth and progression. Our initial focus for this program is NSCLC. We are also exploring other solid tumors that also have the genetic mutations and alterations of interest including gastroesophageal and HNSCC. Because the trial is open-label, we anticipate seeing evidence of clinical activity from the expansion cohorts in the mid-2015.

In mid-2015, we also plan to initiate a combination study of MGCD265 with an EGFR inhibitor in solid tumors, with an initial focus on NSCLC.

We believe that by selecting genetic mutations and alterations that are implicated as oncogenic drivers and that are potently inhibited by MGCD265 we may increase the likelihood of seeing clinical activity earlier in clinical development. We are currently using commercially available diagnostic assays as well as assays developed internally for early clinical use. We are developing companion diagnostics in collaboration with diagnostic platform providers that we plan to use for later stage registration trials and commercialization, if approved.

### MGCD516 - A Novel Multi-targeted Kinase Inhibitor for Solid Tumors

MGCD516 is our second orally-bioavailable, potent, small molecule multi-targeted kinase inhibitor. MGCD516 is a potent inhibitor of closely related RTKs including the Trk, RET and DDR kinase families. We plan to focus our initial development efforts on solid tumors in which genetic mutations and alterations of Trk, RET, or DDR families are implicated as oncogenic drivers with an initial focus on NSCLC. Genetic alterations in Trk, RET, and DDR account for approximately 4% of NSCLC cases, or 7,640 patients annually in the U.S. We also plan to evaluate other tumor types for which the RTK targets of MGCD516 are dysregulated. We are currently evaluating MGCD516 in a Phase 1 trial and MGCD516 is currently in the dose escalation portion of that trial. Once projected clinically active concentrations are achieved, which we anticipate could occur in mid-2015, we plan to initiate expansion cohorts in patients selected for certain genetic alterations (driver mutations) that increase the likelihood that their tumors will respond to single agent MGCD516.

MGCD516 has demonstrated oral bioavailability in preclinical studies, inhibited target-dependent tumor cell growth and survival, and demonstrated broad spectrum antitumor activity in preclinical cancer models including tumor regression in tumor models exhibiting genetic alteration of MGCD516 RTK targets.

## Mocetinostat - A Spectrum-Selective Oral HDAC Inhibitor for Bladder Cancer, DLBCL and FL Patients with Certain Genetic Alterations

### Mocetinostat Overview

Mocetinostat is an orally-bioavailable, spectrum-selective HDAC inhibitor currently in development for the treatment of patients with bladder cancer and NHL, specifically DLBCL and FL, whose tumors have a certain genetic alteration in genes that regulate histone acetylation and that have been shown to increase the sensitivity of their tumor cells to mocetinostat in preclinical models. We are also continuing to evaluate mocetinostat for the first line treatment of patients with MDS in combination with Vidaza, an HMA, although our primary focus is on bladder cancer and DLBCL. We have completed 13 clinical trials with mocetinostat which enrolled approximately 450 patients with a variety of hematologic malignancies and solid tumors. Phase 2 trials in bladder cancer, NHL and MDS are ongoing. The Phase 2 bladder cancer trial is designed to convert to a single-arm registration-enabling study if the initial proof of concept data is sufficient to support an accelerated approval pathway. We anticipate initial proof of concept data in bladder cancer and DLBCL in mid-2015.

We believe that the epigenetic mechanisms of HDAC inhibitors may be important in the treatment of certain cancers and potentially complementary with other epigenetic mechanisms. 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. In particular, alterations of two histone acetyl transferase or HAT genes, CREBBP and EP300, are found in 20 to 30% of patients with DLBCL or bladder cancers. CREBBP and EP300 are implicated in the silencing of selected tumor suppressor genes and which contribute to tumor growth and progression. In an evaluation of over 30 mutant cell lines and 20 xenograft models, those with CREBBP or EP300 mutations were highly responsive to mocetinostat. Because the epigenetic regulation of gene expression is controlled by both DNA methylation and histone modification, we have focused on developing a patient selection strategy based on enrichment of patients exhibiting these genetic alterations.

### Mocetinostat Market Overview

The potential of HDAC inhibitors for the treatment of certain cancers has been validated by the approval of Zolinda and Istodax for the treatment of T-cell lymphoma. Our clinical studies of mocetinostat indicate that it may have promising activity as a single agent in bladder cancer and DLBCL as well as activity in MDS in combination with Vidaza. Mocetinostat single agent responses have been seen in patients with NHL, including DLBCL and FL. In addition, responses to combination therapy have been seen in patients with MDS and AML.

Our initial focus for mocetinostat is on the second line treatment of patients with bladder cancer and the second line or later treatment of patients with DLBCL, estimated to be approximately 9,780 patients annually in the United States.

**Bladder Cancer.** The NCI reports the United States annual incidence of bladder cancer to be 74,690 patients, of which approximately 30%, or 22,400 patients, have metastatic/refractory disease. It is estimated that approximately 20-30% of these metastatic/refractory patients have mutations in either CREBBP or EP300, which are of interest in our development of mocetinostat. Therefore, the annual target patient population in the U.S. for mocetinostat in bladder cancer is approximately 4,480 to 6,720 patients. Treatment of bladder cancer is a high unmet need as there are no approved drugs in the U.S. for second line treatment of bladder cancer.

**DLBCL.** The NCI reports the U.S. annual incidence of NHL to be 70,800 patients of which approximately 30%, or 21,200, are DLBCL. It is estimated that approximately 25% of these DLBCL patients have a mutation in either

CREBBP or EP300 which are of interest in our development of mocetinostat. Therefore, the annual target patient population in the U.S. for mocetinostat in DLBCL is approximately 5,300. While there are other approved agents for DLBCL, there is much room for improvement in clinical outcomes. Mocetinostat has the potential to be the first genetically targeted therapy for DLBCL.

MDS. MDS consists of a group of heterogeneous, clonal hematopoietic stem cell disorders that are characterized by abnormal bone marrow and blood cell development. According to NCI, MDS would be diagnosed in more than 10,000 people annually in the United States. Utilizing Surveillance Epidemiology and End Results data from NCI, Decision Resources estimates the prevalence of MDS to be over 52,000 patients in the United States and over 49,000 patients in the European Union.

Mocetinostat Background

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Histones are protein components of the structural architecture of DNA known as chromatin (chromatin is the material that chromosomes are made of, and is comprised of DNA and histone proteins). Local gene expression activity can be controlled through epigenetic mechanisms by inducing changes in chromatin conformation through chemical modifications of histones. Acetylated histones are associated with a more open configuration of chromatin that is receptive to gene expression signals. In contrast, decreases in histone acetylation result in a more compact structure where gene expression is restricted or suppressed. Tumor suppressor genes serve to regulate cell growth and cell death, but during oncogenesis these tumor suppressor genes may become silenced due to HDAC-dependent decreases in histone acetylation leading to unrestricted growth of tumor cells. HDACs are a family of 11 enzymes (the individual HDAC enzymes are referred to as isoforms) that appear to act as a master regulator of the expression of genes. HDAC inhibitors modulate inappropriate deacetylation of histones to restore normal acetylation patterns as well as tumor suppressor gene expression. Inhibition of HDACs may result in multiple anti-cancer effects such as (1) the inhibition of cancer cell proliferation, (2) the induction of apoptosis (cell death) of cancer cells, (3) improved cell cycle regulation, (4) the induction of tumor suppressor genes, and (5) re-establishing normal histone acetylation activity in cells where mutations or alterations may cause a loss of normal function.

We believe that a key differentiating feature of mocetinostat is its spectrum of activity, targeting HDAC isoforms 1, 2, 3 and 11. We believe that these isoforms, and particularly isoforms 1 and 2, are the most relevant HDAC isoforms in cancer therapy and are also the isoforms most potently inhibited by mocetinostat. Compared to other HDAC inhibitors that have a broader spectrum of activity, the profile of mocetinostat may allow us to inhibit the targets relevant to cancer more potently and thereby potentially demonstrate improved clinical efficacy and reduced side effects.

#### Mocetinostat Clinical Development

Our IND for mocetinostat was submitted in December 2003 and became effective in January 2004. To date, we have evaluated mocetinostat as a monotherapy and in combination with other anticancer agents in approximately 450 patients in Phase 1 and Phase 2 clinical trials with various malignancies, including MDS, HL, NHL (including DLBCL or FL), acute myeloid leukemia, or AML, chronic lymphocytic leukemia and chronic myelogenous leukemia, as well as advanced solid tumors. Through these trials, the safety and tolerability of mocetinostat as a single agent and in combination has been well characterized. The clinical trials showed activity as a single agent in HL and NHL and in combination with Vidaza in MDS and AML. None of these prior trials were conducted in genetically selected patients.

The historical mocetinostat clinical trials are set forth in the following table.

#### CLINICAL TRIALS EVALUATING MOCETINOSTAT

Phase 1 Clinical Trial	Daily dosing regimen (14 days on, 7 days off) Three times weekly (14 days on, 7 days off) Three times weekly (continuously) Twice weekly (continuously)
Phase 2 Monotherapy Clinical Trial	AML/High-risk MDS Relapsed/Refractory NHL (DLBCL, FL) Refractory chronic lymphocytic leukemia Relapsed/Refractory HL
Phase 1/2 Combination Clinical Trial with Vidaza	AML and MDS
Other Clinical Trials	

Phase 1/2 clinical trial of Mocetinostat in Combination with  
Gemcitabine  
Combination of mocetinostat with Vidaza and with Taxotere

#### Pericarditis Finding and Clinical Hold

In July 2008 a prior collaborative partner instituted a voluntary clinical hold to new patient enrollment for mocetinostat, which was accepted by the FDA in August 2008. The voluntary clinical hold was put in place in response to an observation of

pericarditis and pericardial effusion (inflammation of the pericardium, the fibrous sac surrounding the heart, and accumulation of fluid around the heart).

Our complete response to the voluntary clinical hold was accepted by the FDA and the hold was lifted in September 2009. Our response included specific guidance for identifying patients at potential risk for, and guidance to manage patients who develop pericarditis or pericardial effusions. As a result, new patient enrollment in mocetinostat clinical trials will include both the exclusion of patients who are diagnosed with cardiac abnormalities prior to starting mocetinostat therapy (i.e. myocardial infarction, congestive heart failure and pericardial disease) and patient monitoring by electrocardiogram and echocardiography at baseline and while on study. These diagnostic tests are non-invasive and relatively common procedures.

Since we restarted mocetinostat development in 2013 and implemented screening procedures, we have not observed any pericardial effusions or pericarditis in any treatment group.

#### Mocetinostat in Lymphoma

We evaluated the safety and efficacy of single agent mocetinostat in unselected patients with relapsed/refractory DLBCL and FL in a trial starting in 2006. Patients continued treatment until disease progression or prohibitive toxicity. A total of 72 patients were enrolled. On the basis of intent-to-treat analysis, the objective response rate was 17% (7 of 41 patients) in patients with DLBCL and 10% (3 of 31) in patients with FL. Initially, 32 patients began treatment at 110 mg three times weekly (21 with DLBCL and 11 with FL), 37 additional patients were treated with a dose of 85 mg three times weekly (20 with DLBCL and 17 with FL) and 3 FL patients were treated with a dose of 70 mg three times weekly. The most commonly reported adverse events included myelosuppression and fatigue.

We are evaluating opportunities for further development of mocetinostat for the treatment of patients with lymphoma whose tumors exhibit alterations and mutations in the CREBBP and EP300 genes that occur in between 25-30% of DLBCL and FL patients. Based on the single agent responses seen in patients with NHL and preclinical experiments that demonstrate strong single agent activity in tumors that express these genetic alterations in histone acetylation, we believe that this may be a subset of genetically identifiable lymphoma and solid tumor patients more likely to respond to mocetinostat. A Phase 2 trial is currently ongoing designed to select DLBCL and FL patients with CREBBP and EP300 genetic mutations that we believe will make them more responsive to treatment with mocetinostat.

#### Mocetinostat in Bladder Cancer

In the spring of 2014, data was published from The Cancer Genome Atlas, or TCGA, indicating for the first time that the same defects in histone acetylation that are of interest in DLBCL and FL also occur in bladder cancer. While the data is still emerging, it appears that these alterations exist in 20-25% of bladder cancer patients. Our Phase 2 trial in patients with bladder cancer is ongoing in patients with genetic mutations in CREBBP and EP300 that we believe will make them more responsive to treatment with mocetinostat. The Phase 2 study in bladder cancer patients is designed to enable registration if the response rate is sufficiently robust.

#### Mocetinostat in MDS

A Phase 2 trial of mocetinostat in combination with Vidaza in MDS in unselected patients is ongoing. While the study is primarily for safety and to confirm the clinical dose, we are exploring a patient selection strategy for MDS using genetic testing methods.

#### Intellectual Property



## Patents and Proprietary Technology

Our goal is to obtain, maintain and enforce patent protection wherever appropriate for our product candidates, formulations, processes, methods and any other proprietary technologies and operate without infringing on the proprietary rights of other parties, both in the United States and in other countries. Our practice is to actively seek to obtain, where appropriate, intellectual property protection for our current product candidates and any future product candidates, proprietary information and proprietary technology through a combination of patents, protection of proprietary know-how and trade secrets, and contractual arrangements, both in the United States and abroad. However, patent protection may not afford us with complete protection against competitors who seek to circumvent our patents. We also depend upon the skills, knowledge, experience and know-how of our management and research and development personnel as well as that of our advisors, consultants and other contractors. To help protect our proprietary know-how that is not patentable, we seek to put in place appropriate internal policies for the management of confidential information,

and require all of our employees, consultants, advisors and other contractors to enter into confidentiality agreements that prohibit the disclosure of confidential information and which require disclosure and assignment to us of the ideas, developments, discoveries and inventions important to our business.

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, 2014, we own or co-own U.S. patents and patent applications and their foreign counterparts, including 25 issued U.S. patents as reflected in the following table:

Granted and Pending U.S. Patents

Program	Granted (United States)	Pending (United States)
Kinase	14	3
HDAC	11	4
TOTAL	25	7

Kinase - (14 granted U.S. patents; 3 pending U.S. patent applications)

As of December 31, 2014, we have fourteen issued patents and three pending patent applications in the United States covering inhibitor compounds, including MGCD265 and MGCD516, and methods of use of these compounds. Of these issued patents, one covers multiple series of kinase inhibitors and protects MGCD265 generically. Another issued patent, which expires no earlier than 2026, protects a selection of compounds including MGCD265, as well as methods of inhibiting VEGF and HGF receptor signaling, and methods of treating angiogenesis-mediated cell proliferative disease or inhibiting solid tumor growth. Two issued patents cover processes of manufacturing kinase inhibitors such as MGCD265 and MGCD516, and synthetic intermediates required for the production of these inhibitors. Exclusivity arising from our issued patents for MGCD265 extends to at least 2026, including our patents covering the specific composition of matter of MGCD265 (expires 2026, prior to any legal or regulatory extensions, including any patent term extension, that may be available under the Hatch Waxman Act) and the generic class of compounds to which MGCD265 belongs (expires 2025, prior to legal or regulatory extensions, including any patent term extension, that may be available under the Hatch Waxman Act). Another four issued patents cover several distinct classes of compounds. Such coverage includes specific claims to MGCD516, generic coverage of the class of compounds to which MGCD516 belongs, as well as patents covering methods of use of such compounds. Exclusivity arising from our patent protection for MGCD516 extends to at least 2029, prior to legal or regulatory extensions, including any patent term extension that may be available under the Hatch Waxman Act.

Our pending patent applications relating to our kinase inhibitors seek coverage of a broader scope of kinase inhibitors both for oncology and for the treatment of ophthalmic diseases. Methods of use of these inhibitors, such as methods of inhibiting VEGF and HGF receptor signaling, methods of treating angiogenesis-mediated cell proliferative disease or inhibiting solid tumor growth are also being pursued.

HDAC Program - (11 granted U.S. patents; 4 pending U.S. patent applications)

Our patent estate for our HDAC program covers multiple series of HDAC inhibitors, including mocetinostat. This group of patents includes 11 issued patents and 4 pending patent applications in the United States protecting composition of matter and method of use. Two issued patents cover mocetinostat generically and specifically. Exclusivity for mocetinostat extends to 2022 prior to legal or regulatory extensions, including any patent term extension that may be available under the Hatch Waxman Act.

In aggregate, these U.S. patents and patent applications cover the following inventions: novel HDAC inhibitors, including mocetinostat (eleven issued patents and three patent applications), methods of inhibiting HDACs, methods for treating cell proliferative disease or cancer, specific methods for treating colon, lung and pancreatic cancers, and methods for treating polyglutamine expansion diseases (such as Huntington's disease. One pending application claims pharmaceutical compositions comprising a specific HDAC inhibitor and methods of use inhibiting HDACs for treating neurodegenerative disorders.

#### Licensing Agreements

We may enter into license or sub-license agreements when we believe such license is required to pursue a specific program.

## Competition

### Competitors in Oncology - Small Molecule Kinase Inhibitors

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

Our MGCD265 program is attractively positioned in the pipeline of MET-targeted molecules and is characterized by potential advantages including: a unique kinase spectrum including the emerging RTK target Axl; potent inhibition of MET driver mutations which are not inhibited by other small molecule inhibitors due to a different mode of binding to the MET molecule; a lack of activity against over 300 off-target kinases, supporting a favorable safety profile; and excellent tolerability to date in combination with other anti-cancer agents (including chemotherapy), thus optimizing the potential for combination therapy approaches.

Companies with MET inhibitors believed to be in late preclinical or clinical development include, but are not limited to: AbbVie, Inc., Amgen Inc., Exelixis Inc., GlaxoSmithKline PLC, Incyte Corporation, Merck KGaA, Novartis AG, Pfizer Inc., and Sanofi S. A.

Companies with Axl inhibitors in clinical development include, but are not limited to, Exelixis, BergenBio, and GlaxoSmithKline PLC.

### Competitors in Oncology - Mocetinostat Competitors

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., 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 therefore not as selective as molecules like mocetinostat, include but are not limited to: Celgene, Curis Inc., MEI Pharma Inc., Merck, 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.

### Competitors in Oncology - General Competitors

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. Other important competitors, in addition to those mentioned above, include: small and large biotechnology companies, including but not limited to Amgen, Celgene and Exelixis; and specialty and regional pharmaceutical companies and multinational pharmaceutical companies, including but not limited to Abbott Laboratories Inc., Astellas Pharma Inc., AstraZeneca plc, Bayer-Schering Pharmaceutical, Boehringer Ingelheim AG, Bristol-Myers Squibb, Eisai Co. Ltd., Eli Lilly and Company, F. Hoffmann-LaRoche Ltd.,

GlaxoSmithKline, Johnson & Johnson, Merck, Novartis, Pfizer, Sanofi S.A., 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, Compugen Limited, 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.

## Employees

As of December 31, 2014, we had 33 employees located in our offices in San Diego. We also utilize the services of consultants on a regular basis. Twenty employees are engaged in product development activities and 13 are in support administration, including business development. Our operations in Montreal, Quebec ceased in March 2014 and our operations have fully transitioned to San Diego.

## Executive Officers and Directors

The following table sets forth information about our executive officers, directors and key employee as of December 31, 2014.

Name	Age	Position
Charles M. Baum, M.D., Ph.D.	56	President and Chief Executive Officer, Director
Mark J. Gergen	52	Executive Vice President and Chief Operating Officer
Isan Chen, M.D.	52	Executive Vice President and Chief Medical and Development Officer
James Christensen, Ph.D.	46	Senior Vice President and Chief Scientific Officer
Jamie A. Donadio	39	Vice President, Finance
Rodney W. Lappe, Ph.D. <sup>(3)</sup>	60	Chairman of the Board
Michael Grey <sup>(1)(3)</sup>	62	Director
Henry J. Fuchs, M.D. <sup>(2)(3)</sup>	55	Director
Craig Johnson <sup>(1)(2)</sup>	53	Director
William R. Ringo <sup>(1)(2)</sup>	69	Director

(1) Member of the Audit Committee.

(2) Member of the Compensation Committee.

(3) Member of the Nominating and Corporate Governance Committee.

## Executive Officers

Charles M. Baum, M.D., Ph.D. has served as our President and Chief Executive Officer and member of our Board of Directors since November 2012. From June 2003 to September 2012, he was at Pfizer as Senior Vice President for Biotherapeutic Clinical Research within Pfizer's Worldwide Research & Development division and as Vice President and Head of Oncology Development and Chief Medical Officer for Pfizer's Biotherapeutics and Bioinnovation Center. From 2000 to 2003, he was responsible for the development of several oncology compounds at Schering-Plough Corporation (acquired by Merck). His career has included academic and hospital positions at Stanford University and Emory University, as well as positions of increasing responsibility within the pharmaceutical industry at SyStemix, Inc. (acquired by Novartis AG), G.D. Searle & Company (acquired by Pfizer), Schering-Plough Corporation (acquired by Merck) and Pfizer. Dr. Baum currently serves on the board of directors of Array BioPharma. Dr. Baum received his M.D. and Ph.D. (Immunology) degrees from Washington University School of Medicine in St. Louis, Missouri and completed his post-doctoral training at Stanford University.

Dr. Baum's experience in the pharmaceutical industry provides our Board of Directors with subject matter expertise. In addition, through his position as Chief Medical Officer for Pfizer's Biotherapeutics and Bioinnovation Center, Dr. Baum has acquired the operational expertise, which we believe qualifies him to serve on our Board of Directors. Mark J. Gergen has served as our Executive Vice President and Chief Operations Officer since February 2013. From September 2006 to November 2012, he was Senior Vice President, Corporate Development for Amylin Pharmaceuticals, Inc., or Amylin. Prior to Amylin, Mr. Gergen was Executive Vice President of CardioNet, Inc., and he previously served as Chief Financial and Development Officer and later Chief Restructuring Officer of Advanced Tissue Sciences, Inc. From August 1994 to June 1999, he was Division Counsel at Medtronic, Inc. Mr. Gergen received a B.A. in Business Administration from Minot State University and a J.D. from the University of Minnesota Law School.

Isan Chen, M.D. has served as our Executive Vice President and Chief Medical and Development Officer since September 2013. Dr. Chen is board certified in Internal medicine, hematology and medical oncology with more than 15 years of experience in oncology and clinical trials from first-in-humans through global registrational studies. He has experience in oncology clinical development and interactions with regulatory agencies in the United States and Europe. He was most recently the Chief Medical Officer of Aragon Pharmaceuticals, which was acquired by Johnson & Johnson in July of 2013. At Aragon Pharmaceuticals, Dr. Chen was responsible for the clinical development strategy of all the company's programs, including prostate and breast cancer. Prior to Aragon Pharmaceuticals, Dr. Chen served as Vice President of tumor strategy in the oncology business unit at Pfizer. In

addition he was the clinical lead for Sutent, a multiple kinase inhibitor, for the treatment of RCC, an indication in which the drug secured FDA approval in 2006. He was also the clinical lead for the Phase 1 studies of crizotinib and CDK 4/6 inhibitor palbociclib. Dr. Chen completed his hematology/oncology fellowship at University of California, San Diego. Before joining Pfizer, Dr. Chen practiced medicine as a staff physician at City of Hope Medical Center and later as an assistant professor at the University of Texas, M.D. Anderson Cancer Center.

James Christensen, Ph.D. has served as our Senior Vice President, and Chief Scientific Officer since January 2014 and served as our Vice President, Research from June 2013 through January 2014. Prior to joining us, he held various positions at Pfizer from 2003 to 2013, the most recent of which was Senior Director of Oncology Precision Medicine in the Oncology Research Unit. While Dr. Christensen joined Pfizer in 2003 and his responsibilities there included leading nonclinical research efforts for oncology programs including sunitinib malate research activities and leading the nonclinical and translational biology efforts for other research and development programs including crizotinib.

Dr. Christensen participated as a member of the Cancer Research or Oncology Research Unit leadership team from 2005 to 2013. Prior to 2003, Dr Christensen was a Group Leader on the Preclinical Research and Exploratory Development team at SUGEN, Inc., which was acquired by Pharmacia Corporation, now owned by Pfizer. Dr. Christensen began his career in 1998 at Warner Lambert, now owned by Pfizer, with research focus in RTK biology and RTK pathway biomarker development in the oncology therapeutic area. Dr. Christensen participates on the editorial boards for Cancer Research and Molecular Cancer Therapeutics. Dr. Christensen received a Ph.D. in molecular pharmacology from North Carolina State University with dissertation research directed toward characterization of mechanisms of apoptosis dysregulation during the process of carcinogenesis.

Jamie A. Donadio has served as our Vice President, Finance since March 2013. Prior to joining us, Mr. Donadio was at Amylin Pharmaceuticals from April 2001 through January 2013. From November 2011 to January 2013, Mr. Donadio served as Senior Director of Finance at Amylin. From December 2010 to November 2011, he served as Director of Corporate Financial Planning and Analysis at Amylin. From March 2007 to December 2010 he served as Director of SEC Reporting and from April 2001 to March 2007 he held various corporate accounting roles at Amylin. From December 2000 to April 2001, Mr. Donadio was senior accountant at Novatel Wireless, Inc. From August 1997 to December 2000, Mr. Donadio was with Ernst & Young LLP, last serving as an audit senior. Mr. Donadio holds a B.S. in Accounting from Babson College and is a certified public account (inactive) in the State of California.

#### Non-Employee Directors

Henry J. Fuchs, M.D. has served as a member of our Board of Directors since February 2012. Since March 2009, Dr. Fuchs has served as the Executive Vice President and Chief Medical Officer of BioMarin Pharmaceutical Inc. From September 2005 to December 2008, Dr. Fuchs was Executive Vice President and Chief Medical Officer of Onyx Pharmaceuticals, Inc. From 1996 to 2005, Dr. Fuchs served in multiple roles of increasing responsibility at Ardea Biosciences, Inc., first as Vice President, Clinical Affairs, then as President and Chief Operating Officer, and finally as Chief Executive Officer. From 1987 to 1996, Dr. Fuchs held various positions at Genentech Inc. Dr. Fuchs serves on the Board of Directors of Genomics Health, Inc. and was on the Board of Directors of Ardea Biosciences, Inc. from 1996 until its acquisition by AstraZeneca PLC in 2012. Dr. Fuchs received a B.A. in Biochemical Sciences from Harvard University, and an M.D. from George Washington University.

We believe that Dr. Fuchs' experience as an executive and his breadth of knowledge and valuable understanding of the pharmaceutical industry qualify him to serve on our Board of Directors.

Michael Grey has served as a member of our Board of Directors since November 2014. Mr. Grey currently serves as Chief Executive Officer and Chairman of Reneo Pharmaceuticals. He recently served as President and Chief Executive Officer of Lumena Pharmaceuticals, Inc., a privately-held biotechnology company before it was acquired by Shire. He is also serving as a Venture Partner with Pappas Ventures, a life sciences venture capital firm, since January 2010. Between January and September 2009, he served as President and Chief Executive Officer of Auspex Pharmaceuticals, Inc., a private biotechnology company. From January 2005 until its acquisition in August 2008, Mr. Grey was President and Chief Executive Officer of SGX Pharmaceuticals, Inc., a public biotechnology company, where he previously served as President from June 2003 to January 2005 and as Chief Business Officer from April 2001 until June 2003. Prior to joining SGX Pharmaceuticals, Inc., Mr. Grey acted as President, Chief Executive Officer and Board member of Trega Biosciences, Inc., a biotechnology company. From November 1994 to August



1998, Mr. Grey was the President of BioChem Therapeutic, Inc., the pharmaceutical operating division of BioChem Pharma, Inc. During 1994, Mr. Grey served as President and Chief Operating Officer for Ansan, Inc., a pharmaceutical company. From 1974 to 1993, he served in various roles with Glaxo, Inc. and Glaxo Holdings, plc, culminating in the position of Vice President, Corporate Development. Mr. Grey is currently a director of Horizon Pharma, Inc., a public pharmaceutical company, and Selventa, Inc., a healthcare company. Mr. Grey previously served on the board of directors of two public companies during the past five years: IDM Pharma, Inc. (from 1999 to 2009) and Achillion Pharmaceuticals, Inc. (from 2001 to 2010). He received a B.Sc. in chemistry from the University of Nottingham, United Kingdom.

Based on Mr. Grey's experience as an executive in the biopharmaceutical industry and his breadth of knowledge and valuable understanding of the pharmaceutical industry qualify him to serve on our Board of Directors.

Craig Johnson has served as a member of our Board of Directors since September 2013. Mr. Johnson serves on the boards of directors for several life science companies. He is currently a director for Heron Therapeutics, Inc., a NASDAQ-listed specialty pharmaceutical company, as well as La Jolla Pharmaceutical Company, a NASDAQ-listed biopharmaceutical company. Mr. Johnson also served as a past director of Adamis Pharmaceuticals Corporation, a NASDAQ-listed biopharmaceutical company, from 2011 to 2014, as well as Ardea Biosciences, Inc., a NASDAQ-listed biotechnology company, from 2008 until its sale to AstraZeneca PLC in 2012. From 2011 to 2012 he was Chief Financial Officer of PURE Bioscience, Inc., and from 2010 to 2011 he was Senior Vice President and Chief Financial Officer of NovaDel Pharma Inc. Mr. Johnson served as Vice President and Chief Financial Officer of TorreyPines Therapeutics, Inc. from 2004 until its sale to Raptor Pharmaceuticals Corp. in 2009, and then as Vice President of a wholly-owned subsidiary of Raptor Pharmaceutical Corp. from 2009 to 2010. He held several positions, including Chief Financial Officer and Senior Vice President of Operations, at MitoKor, Inc. from 1994 to 2004. Prior to 1994, Mr. Johnson held senior financial positions with several early-stage technology companies, and also practiced as a Certified Public Accountant with Price Waterhouse. Mr. Johnson received his B.B.A. in accounting from the University of Michigan-Dearborn.

We believe Mr. Johnson's leadership and experience and skills in accounting and finance qualify him to serve on our Board of Directors.

Rodney Lappe, Ph.D. has served as a member of our Board of Directors since June 2012, and as Chairman of the Board since July 2013. Since January 2012, Dr. Lappe has served as the Senior Vice President of Tavistock Life Sciences, a private investment firm. From January 2004 to December 2011, Dr. Lappe was Group Senior Vice President, Pfizer Worldwide Research and Development and Chief Scientific Officer for CovX in San Diego, California. Dr. Lappe joined Pfizer with the CovX acquisition in 2008. From 2000 to 2002, Dr. Lappe served as Vice President for cardiovascular and metabolic diseases at Pharmacia. He was also site leader for Pharmacia in St. Louis. Prior to joining Pharmacia, he held positions of increasing responsibility with Wyeth, Rorer Central Research, CIBA Geigy and Searle Pharmaceuticals. Dr. Lappe received his B.A. from Blackburn College and his Ph.D. in Pharmacology from Indiana University.

We believe Dr. Lappe's extensive experience managing pharmaceutical and biotech companies bring important strategic insight and qualifies him to serve on our Board of Directors.

William Ringo, has served as a member of our Board of Directors since March 2014. Mr. Ringo has over 40 years of experience in the pharmaceutical and biotechnology sectors. Currently, he serves as a senior advisor with investment bank Barclays Capital and also serves as a strategic advisor with Sofinnova Ventures. Previously, Mr. Ringo was senior vice president of strategy and business development for Pfizer before his retirement in April 2010. He spent nearly 30 years with Eli Lilly and Company, serving in numerous executive roles, including product group president for oncology and critical care, president of internal medicine products, president of the infectious disease business unit and vice president of sales and marketing for U.S. pharmaceuticals. He has also served as president and CEO of Abgenix, an oncology-focused antibody company that was purchased by Amgen. He currently serves on the board of directors of Sangamo BioSciences, Immune Design Corp, Five Prime Therapeutics, Dermira, Assembly Biosciences and BioCrossroads, an Indiana initiative and public-private collaboration focused on growing, advancing and investing in life sciences. He also recently served on the board of directors for Onyx Pharmaceuticals until its acquisition by Amgen in 2013. Mr. Ringo earned a B.S. in business administration and an M.B.A. from the University of Dayton.

We believe that Mr. Ringo's experience as an executive and his breadth of knowledge and valuable understanding of the pharmaceutical industry qualify him to serve on our Board of Directors.

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 \$26.1 million, \$19.8 million, and \$15.1 million for the years ended December 31, 2014, 2013 and 2012, respectively. In February 2015 we completed a public offering of our common stock that generated estimated net proceeds of \$48.2 million. We believe that our current cash and cash equivalents and short-term investments together with the estimated net proceeds from the February 2015 common stock offering will sustain our operations through the third quarter of 2016. Pursuant to our current plans, we do not anticipate initiating Phase 3 clinical trials with mocetinostat until data from our Phase 2 clinical trials is available and until additional financing or the establishment of a collaboration for late-stage development. We have based these estimates on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect. 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 FDA or any foreign regulatory agency, such as the European Medicines Agency, or 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. Any delay resulting from such further or repeat studies or trials could also result in the need for additional financing. 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 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, or 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 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, 2014, 2013, and 2012 were \$43.7 million, \$52.9 million, and \$20.3 million respectively. As of December 31, 2014, we had an accumulated

deficit of \$242.1 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;
- entering into collaboration and license agreements;
- 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 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.

We may incur losses associated with foreign currency fluctuation.

Our headquarters were previously located in Canada and many of our material contracts were entered into in Canada. A significant portion of our expenditures are in foreign currencies, most notably in Canadian dollars; therefore, we are subject to foreign currency fluctuations which may, from time to time, impact (positively or negatively) our financial position and results of operations. Exchange rates can fluctuate significantly and cannot be easily predicted; thus, we may experience significant shifts in currency exchange variances in the future. We maintain bank accounts in both Canadian dollars and U.S. dollars and do not hedge our positions. Our functional currency at December 31, 2014 and 2013 was the U.S. dollar. Prior to January 1, 2013 our functional currency was the Canadian dollar.

As a public company in the United States, 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, or the 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, or 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 the trading price of our common stock to fall dramatically.

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 lead to a lower share price. When we are no longer an “emerging growth company” (as defined in the Exchange Act or the Securities Act of 1933, as amended, or 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.

We and our independent registered public accounting firm have identified a material weakness in our internal controls that is described in greater detail in Item 9A-Controls and Procedures. We have implemented measures designed to improve our internal control over financial reporting that successfully remediated the control deficiencies that led to our material weakness. We cannot guarantee that the measures we have taken to date, or any measures we may take in the future, will be sufficient to avoid potential future material weaknesses. 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, additional significant deficiencies or material weaknesses may have been identified. If we are unable to successfully remediate any significant deficiency or material weakness in our internal control over financial reporting, or identify any additional significant deficiencies or material weaknesses that may exist, 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.

We will incur significant increased costs as a result of operating as a U.S. public company and continuing to be a Canadian “reporting issuer.”

Although we de-listed from the TSX effective as of July 26, 2013, we will continue to be subject to Canadian reporting obligations until we meet certain prescribed thresholds which would allow us to apply to cease being a Canadian “reporting issuer.” We may incur significant additional accounting, reporting and other expenses in order to maintain our listing on The NASDAQ Capital Market, and fulfill our obligations as a Canadian “reporting issuer.” As a U.S. listed public company, we incur significant additional legal, accounting and other expenses that we did not incur as a company listed on the TSX. 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 will remain an emerging growth company until the earliest of (1) the end of the fiscal year in which the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the end of the second fiscal quarter, (2) the end of the fiscal year in which we have total annual gross revenue of \$1 billion or more during such fiscal year, (3) the date on which we issue more than \$1 billion in non-convertible debt in a three-year period, or (4) 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.

## 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. MGCD265 is currently in a Phase 1b clinical trial, mocetinostat is currently in Phase 2 clinical trials and MGCD516 is in a Phase 1 clinical trial. Each of our product candidates will require 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. 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 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 filing of any new drug applications, or 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 future collaborators' ability to obtain regulatory approval of 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, or 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 adverse 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.

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 are expensive, we may seek to enter into 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 be 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 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 each of MGCD265, MGCD516 and mocetinostat is to identify patients or types of tumors that express specific genetic markers, which 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 these product candidates. 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 any of our product candidates.

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 adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;

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 may not be able to obtain an Special Protocol Assessment ("SPA") prior to initiating Phase 3 clinical trials of mocetinostat for MDS. Even if obtained, an SPA would not guarantee any particular outcome from regulatory review.

If we pursue Phase 3 development of mocetinostat for MDS, we would first plan to submit an SPA to the FDA. The FDA's SPA process creates a written agreement between the sponsoring company and the FDA regarding clinical trial design and other clinical trial issues that can be used to support approval of a product candidate. The SPA is intended to provide assurance that if the agreed upon clinical trial protocols are followed and the clinical trial endpoints are achieved, the data may serve as the primary basis for an efficacy claim in support of an NDA. However, SPA agreements are not a guarantee of an approval of a product candidate or any permissible claims about the product candidate. In particular, SPAs are not binding on the FDA if previously unrecognized public health concerns arise during the performance of the clinical trial, if other new scientific concerns regarding product candidate safety or efficacy arise, or if the sponsoring company fails to comply with the agreed upon clinical trial protocols. We cannot guarantee that we will be able to obtain an SPA if we pursue Phase 3 development of mocetinostat for MDS or that an SPA, if obtained, would ultimately aid in obtaining regulatory approval.

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. 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 or 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, or 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 recruiting suitable patients to participate in a trial;
- 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.

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 relatively 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. For example, 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, 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.

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 adverse 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 the adoption of 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, 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 Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or 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 candidate 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. 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. 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, PPACA, became law in the United States. PPACA substantially changes the way healthcare is financed by both governmental and private insurers and significantly affects the healthcare industry. The provisions of PPACA of importance to our product candidates include the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, to 23.1% and 13.0% of the average manufacturer price for most branded and generic drugs, respectively;

- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;

- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for a manufacturer's outpatient drugs to be covered under Medicare Part D;

- extension of a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the federal poverty level beginning in 2014, thereby potentially increasing a manufacturer's Medicaid rebate liability;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;

- new requirements under the federal Open Payments program and its implementing regulations (as described below);

- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since PPACA 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 will stay in effect through 2024 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 recently enacted Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Among the requirements of this new legislation, manufacturers will be required to provide certain information regarding the drug products to individuals

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

the Middle Class Tax Relief and Job Creation Act of 2012 required the Centers for Medicare & Medicaid Services, or CMS, to reduce the Medicare clinical laboratory fee schedule by 2% in 2013, which revised schedule served as a base for 2014 and will be the base for future years. Beginning January 1, 2016, there will be major changes to the payment formula under the Medicare Clinical Laboratory Fee Schedule, or CLFS. Under the Protecting Access to Medicare Act of 2014, or PAMA, which was signed to law in April 2014, clinical laboratories must report laboratory test payment data for each Medicare-covered clinical diagnostic lab test that it furnishes during a time period to be defined by future regulations. The reported data must include the payment rate (reflecting all discounts, rebates, coupons and other price concessions) and the volume of each test that was paid by each private payor (including health insurance issuers, group health plans, Medicare Advantage plans and Medicaid managed care organizations). Beginning in 2017, the Medicare payment rate for each clinical diagnostic lab test will be equal to the weighted median amount for the test from the most recent data collection period. The payment rate will apply to laboratory tests furnished by a hospital laboratory if the test is separately paid under the hospital outpatient prospective payment system. Levels of reimbursement may be impacted by 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 our companion diagnostics, 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: AbbVie, Inc., Amgen Inc., Exelixis Inc., GlaxoSmithKline PLC, Incyte Corporation, Merck KGaA, Novartis AG, Pfizer Inc., and Sanofi S. A. 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; Compugen Limited; Exelixis; GlaxoSmithKline; Novartis; and Pfizer. 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. 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.

Even though we have obtained orphan drug designation for mocetinostat for MDS and Diffuse Large B-Cell Lymphoma, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. In June 2014, the FDA granted orphan drug status to mocetinostat for the treatment of patients with MDS in the United States, and in August 2014 the FDA granted orphan drug status to mocetinostat for the treatment of patients with Diffuse Large B-Cell Lymphoma in the United States. Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same drug for that same indication for that time period. We can provide no assurance that another drug will not receive marketing approval prior to our product candidates. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA can subsequently approve another drug containing the same active ingredient for the same condition before the expiration of the seven year exclusivity period if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, the EMA may deny marketing approval for a product candidate if it determines such product candidate is structurally similar to an approved product for the same indication.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

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, Mark J. Gergen, our Executive Vice President and Chief Operations Officer, Isan Chen, M.D., our Executive Vice President and Chief Medical and Development

Officer, James Christensen, Ph.D. our Chief Scientific Officer, and Jamie A. Donadio, our Vice President of Finance, 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 or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid;

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;

the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or 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;



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 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 the 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 which began in September 2014; 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. In addition, recent healthcare reform legislation has strengthened these laws. For example, PPACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute such that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. Moreover, PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act. 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 and criminal penalties, damages, fines, imprisonment, 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, it 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;
- a diversion of management's time and our resources;

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- 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 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 law suits 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.

### 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 a claim, 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 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 restrict 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 regulatory authority will approve of any of our trademarks or will not request reconsideration of one of our trademarks 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, non-disclosure and non-compete 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 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 therefrom 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 restrict 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

could be reversed 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 obtained 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. 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. 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. 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 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, or

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 manufacture 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 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, following our common stock offering of 2.6 million shares which completed on February 3, 2015, our stockholders and their affiliates who owned more than 5% of our outstanding common stock collectively owned approximately 62% of our outstanding common stock. Baker Bros. Advisors, L.L.C., or Baker Brothers, and Tavistock Life Sciences Co., or Tavistock, and their affiliates collectively own approximately 36% of our outstanding common stock. In addition, in conjunction with certain financing transactions, we granted to Baker Brothers and Tavistock 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 Tavistock 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, Tavistock or any of 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, or the 2013 Plan, and our 2013 Employee Stock Purchase Plan, or the ESPP, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. 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, or 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, or NOLs, and other pre-change U.S. tax attributes (such as

research tax credits) to offset its post-change income may be limited. We 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 9363 Towne Centre Drive, San Diego, California 92121 where we occupy approximately 6,800 square feet of office space. The term of our sublease at Towne Centre Drive, San Diego expires in the first quarter of 2015. In June 2014, we entered into a lease for approximately 18,000 square feet of office space, which will serve as the Company's new corporate headquarters, replacing the current facilities. The lease will commence in three phases, with 2,300 square feet of space which commenced on July 1, 2014, 14,000 square feet of space becoming available in the first quarter of 2015 and the final 1,600 square feet of space becoming available in the first quarter of 2016. The new lease expires on January 31, 2018. We believe that our existing and upcoming 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, or TSX.

On March 6, 2015, the last reported sale price for our common stock on The NASDAQ Capital Market was \$25.08 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 and the TSX for the period indicated.

	Stock Exchange	High	Currency	Low	Currency
Year Ended December 31, 2014					
Fourth Quarter	The NASDAQ Capital Market*	\$ 19.90	USD	\$ 13.69	USD
Third Quarter	The NASDAQ Capital Market*	\$ 21.58	USD	\$ 15.59	USD
Second Quarter	The NASDAQ Capital Market*	\$ 23.75	USD	\$ 15.86	USD
First Quarter	The NASDAQ Capital Market*	\$ 25.97	USD	\$ 16.50	USD
Year Ended December 31, 2013					
Fourth Quarter	The NASDAQ Capital Market*	\$ 20.90	USD	\$ 15.00	USD
Third Quarter (from July 15, 2013 through September 30, 2013)	The NASDAQ Capital Market*	\$ 17.24	USD	\$ 7.00	USD
Third Quarter (from July 1, 2013 through July 14, 2013)	TSX**	\$ 7.20	CAD	\$ 6.80	CAD
Second Quarter	TSX**	\$ 8.50	CAD	\$ 3.50	CAD
First Quarter	TSX**	\$ 10.00	CAD	\$ 6.50	CAD

\*Prices quoted for The NASDAQ Capital Market are in U.S. dollars.

\*\* Prices quoted for the TSX are in Canadian dollars.

As of March 6, 2015, we had 15 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

None.

#### Use of Proceeds

We commenced our first public offering in the United States pursuant to a registration statement on Form S-1 (File No. 333-191544) that was declared effective by the SEC on October 23, 2013 and registered an aggregate of 3,250,000 shares of our common stock for sale to the public at price of \$17.50 per share for an aggregate offering price of approximately \$56.9 million. On October 29, 2013, we completed the offering. On November 27, 2013 the underwriters exercised their option to purchase an additional 87,500 shares of our common stock at a price of \$17.50 per share and an aggregate additional offering price of approximately \$1.5 million. Jefferies LLC and Leerink Swann LLC acted as joint book-running managers for the offering, and Piper Jaffray & Co. served as co-manager for the offering.

The underwriting discounts and commissions connected with the offering totaled approximately \$3.5 million. We incurred additional costs of approximately \$0.7 million in offering expenses, which when added to the underwriting discounts and commissions paid by us, amounts to total fees and costs of approximately \$4.2 million. Thus, net offering proceeds to us, after deducting underwriting discounts and commissions and offering costs, were \$54.2 million. No offering costs were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

As of December 31, 2014 we have used approximately \$25.1 million of these funds for preclinical and clinical development of our two lead kinase programs, MGCD265 and MGCD516, and our HDAC inhibitor, mocetinostat and related administrative support. We plan to use the remaining net proceeds from our public offering to fund our ongoing and planned clinical and dose confirmation trials for our lead product candidates and for research and development activities, working capital and other general corporate purposes. Our expected use of net proceeds from our public offering represents our current intentions based upon our



present plans and business condition. We cannot predict with certainty all of the particular uses for our current funds, or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual use of these funds will vary depending on numerous factors, including our ability to obtain additional financing, the relative success and cost of our research, preclinical and clinical development programs, and the amount and timing of additional revenues. As a result, our management will have broad discretion in the application of these funds, and investors will be relying on our judgment regarding the application of the net proceeds of the offering.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

None.

#### Item 6. Selected Consolidated Financial Data

Please read the following selected financial data in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the Consolidated Financial Statements and related notes included elsewhere in this annual report on Form 10-K.

Consolidated Statements of Operations and Comprehensive Loss	Year Ended December 31,			
	2014	2013	2012	2011
	(in thousands, except share and per share amounts)			
Revenue				
Collaboration, contract and license revenue	\$—	\$—	\$—	\$3,144
Total revenue	—	—	—	3,144
Expenses				
Research and development	26,071	19,797	15,081	8,891
General and administrative	12,699	11,177	5,417	4,340
Restructuring costs	334	1,025	—	—
Total operating expenses	39,104	31,999	20,498	13,231
Loss from operations	(39,104)	(31,999)	(20,498)	(10,087)
Other income (expense), net	(77)	(1,084)	251	309
Change in fair value of warrant liability*	(4,517)	(19,799)	—	—
Loss before income taxes	(43,698)	(52,882)	(20,247)	(9,778)
Income tax benefit (expense)	—	23	(39)	—
Net loss	\$(43,698)	\$(52,859)	\$(20,286)	\$(9,778)
Unrealized gain (loss) on available-for-sale investments	14	(13)	—	—
Comprehensive loss	\$(43,684)	\$(52,872)	\$(20,286)	\$(9,778)
Basic and diluted net loss per share	\$(3.24)	\$(4.78)	\$(3.00)	\$(1.98)
Weighted average number of shares used in computing net loss per share, basic and diluted	13,483,467	11,057,040	6,762,985	4,944,184

\*Beginning January 1, 2013, the Company reclassified common stock warrants issued in 2011 and 2012 from Stockholders' Equity to current liability due to a January 1, 2013 change in the Company's functional currency. The warrants were recorded at fair value (estimated using the Black-Scholes option-pricing model) and adjusted to their estimated fair value at each reporting date. The increases or decreases in the fair value of such warrants were recorded as a change in fair value of warrant liability in the consolidated statements of operations and comprehensive loss. All the warrants were amended during the second half of 2014 to allow for the warrants to be denominated in U.S. Dollars, at which point, they were reclassified into stockholder's equity and do not require ongoing fair value revisions.

Consolidated Balance Sheet Data	December 31,			
	2014	2013	2012	2011
	(in thousands)			
Cash, cash equivalents and short-term investments	\$29,303	\$62,070	\$36,983	\$28,445
Working capital	27,261	25,563	33,989	26,711
Total assets	33,479	64,537	39,801	31,082
Accumulated deficit	(242,089)	(198,391)	(140,491)	(120,205)
Total stockholders' equity	28,062	25,885	34,416	27,305

## 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.

### Company Overview

Mirati Therapeutics, Inc. ("Mirati") is a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. We focus our development programs on drugs intended to treat specific genetically defined and identified cancer patients with unmet needs. Our pipeline consists of three product candidates: MGCD265, MGCD516 and mocetinostat. MGCD265 and MGCD516 are orally-bioavailable, spectrum-selective kinase inhibitors with distinct target profiles that are in development to treat patients with non-small cell lung cancer, or NSCLC, and other solid tumors including squamous cell carcinoma of the head and neck, or HNSCC. MGCD265 is in Phase 1b clinical development and MGCD516 is in Phase 1 clinical development in the dose escalation phase. Mocetinostat is an orally-bioavailable, spectrum-selective histone deacetylase, or HDAC, inhibitor currently in Phase 2 development. Mocetinostat is being developed for the second line treatment of patients with bladder cancer and non-hodgkins lymphoma, or NHL, specifically focusing on diffuse large B-cell lymphoma, or DLBCL, and follicular lymphoma, or FL. DLBCL and FL tumors have a genetic alteration in one of two genes that have been shown to increase the sensitivity of their tumor cells to mocetinostat in preclinical models. Our development goals for 2015 include demonstrating initial proof of concept for MGCD265 in NSCLC by mid-2015, and if successful, initiating a single-arm registration study by the end of 2015; achieving a dose for MGCD516 that potentially inhibits the targeted genetic alterations by the first half of 2015, and, if successful, initiating dose expansion cohorts in selected patients in mid-2015; and demonstrating initial proof of concept for mocetinostat in bladder cancer and DLBCL by mid-2015, which, if successful, could enable the initiation of single agent registration trials.

We were incorporated under the laws of the State of Delaware on April 29, 2013 as Mirati Therapeutics, Inc. On May 8, 2013, we entered into a plan of arrangement with MethylGene, Inc., or MethylGene Canada, pursuant to which MethylGene Canada became our wholly owned subsidiary and all of its shareholders became proportionate shareholders of ours.

### Liquidity Overview

At December 31, 2014, we had \$29.3 million of cash, cash equivalents and short-term investments compared to \$62.1 million at December 31, 2013. In February 2015, we completed a follow-on offering of common stock for net proceeds of approximately \$48.2 million. We believe that our current cash, cash equivalents and short-term investments together with the net proceeds from the February 2015 common stock offering will be sufficient to fund our currently planned operations through the third quarter of 2016. 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 previous 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.”

We have incurred losses in each year since our inception. Our net losses were \$43.7 million, \$52.9 million, and \$20.3 million for the years ended December 31, 2014, 2013 and 2012, respectively. As of December 31, 2014, we had an accumulated

deficit of \$242.1 million. Substantially all of our operating losses resulted from expenses incurred in connection with our product development programs, research efforts and related general and administrative support associated with our operations.

#### 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, or 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.

#### Warrant Liability

Beginning January 1, 2013, we reclassified common stock warrants issued in 2011 and 2012 from stockholders' equity to current liability due to a January 1, 2013 change in our functional currency. The warrants were recorded at fair value (estimated using the Black-Scholes option-pricing model) and adjusted to their estimated fair value at each reporting date. The increases or decreases in the fair value of such warrants were recorded as a change in fair value of warrant liability in the consolidated statements of operations and comprehensive loss. All the warrants were amended during the second half of 2014 to allow for the warrants to be denominated in U.S. Dollars, at which point, they were reclassified into stockholder's equity and do not require ongoing fair value adjustments.

#### Share-Based Compensation

We have a stock option compensation plan in which the fair value of stock options granted is determined at the date of the grant using the Black-Scholes option-pricing model and is expensed over the vesting period of the options. Stock compensation is recognized using the graded accelerated vesting method. In determining the expense, we deduct the number of options that are expected to be forfeited at the time of a grant and revise this estimate, if necessary, in subsequent periods if actual forfeitures differ from those estimated. Any amounts paid by employees on exercise of the stock options and subsequent purchase of stock are credited to common stock.

The determination of the fair value of share-based compensation awards utilizing the Black-Scholes model is affected by our stock price and a number of assumptions, including but not limited to expected stock price volatility over the term of the awards and the expected term of stock options. Changes in the assumptions can materially affect the fair value estimates. For example, an increase in the underlying stock price results in a significant increase in the Black-Scholes option-pricing, which includes estimates such as expected term, expected volatility and interest rates.

#### Financial Operations Overview

Research and Development Expenses

Research and development expenses consist primarily of:

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- 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;
- contractual obligations for clinical development, clinical sites, manufacturing and scale-up, and formulation of clinical drug supplies; and
- costs for facilities and amortization of equipment.

We record research and development expenses as incurred. 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 MGCD265, MGCD516 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 MGCD265, MGCD516, mocetinostat or any of our preclinical programs into 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 rent and utilities, travel expenses and professional fees for auditing and tax services.

#### Results of Operations

##### Comparison of the Years Ended December 31, 2014 and 2013

The following table summarizes our results of operations for the year ended December 31, 2014 and 2013 (in thousands):

	Year Ended December 31,		Increase
	2014	2013	(Decrease)
Research and development expenses	\$26,071	\$19,797	\$6,274
General and administrative expenses	12,699	11,177	1,522
Restructuring costs	334	1,025	(691)
Other income/(expense), net	(77)	(1,084)	1,007
Change in fair value of warrant liability	(4,517)	(19,799)	15,282

#### Research and Development Expenses

Our research and development efforts during the years ended December 31, 2014 and 2013 were focused primarily on our oncology programs, including our two lead kinase programs, MGCD265 and MGCD516, and our HDAC inhibitor program, mocetinostat. The following table summarizes our research and development expenses, in thousands:

	Year Ended December 31,		Increase (Decrease)
	2014	2013	
Third-party development expense:			
MGCD265	\$7,273	\$6,588	\$685
MGCD516	2,932	2,495	437
mocetinostat	4,507	2,580	1,927
MGCD290*	123	1,629	(1,506 )
Total third-party development expense	14,835	13,292	1,543
Internal research and development expense	11,236	7,336	3,900
Research and development expense, gross	26,071	20,628	5,443
Less: Investment tax credits	—	(831 )	831
Research and development expense	\$26,071	\$19,797	\$6,274

\*Development of MGCD290 ceased in early 2013

For the year ended December 31, 2013, reclassifications were made to certain research and development program expenses line items to conform to the current presentation. These reclassifications had no impact on total research and development expenses.

Research and development expenses for the year ended December 31, 2014 were \$26.1 million compared to \$19.8 million during the year ended December 31, 2013. The increase of \$6.3 million for the year ended December 31, 2014 primarily relates to an increase in third-party development expense of \$1.5 million, an increase of internal research and development expense of \$3.9 million and the absence of investment tax credits, or ITCs, of \$0.8 million during 2014. The increase in third-party development expense relates to an increase in expenses associated with our ongoing clinical trials for our oncology candidates, MGCD265, MGCD516 and mocetinostat and related manufacturing expenses. The increase in internal research and development expense, which includes employee salaries and related expense, facilities expense and early discovery costs, is due to an increase in salaries and related expense, which is largely due to increased stock based compensation expense. Prior to 2014, the Company was eligible to claim ITCs from a Canadian provincial tax authority due to its research and development operations performed within Canada. As a result of our relocation from Montreal, Canada to the United States, the Company is no longer eligible for these ITCs. The aforementioned increased costs are offset by a decrease in costs for MGCD290, which we are no longer developing internally, and the absence of costs incurred in 2013 associated with management changes made during 2013.

#### General and Administrative Expenses

General and administrative expenses for the year ended December 31, 2014 were \$12.7 million compared to \$11.2 million for the same period in 2013. The increase of \$1.5 million for the year ended December 31, 2014 is the result of an increase in salaries and related expense (which is primarily the result of higher stock based compensation costs), increased legal and consulting costs and increased costs for accounting, tax and insurance. These increased costs largely reflect our status as a U.S. public company. Partially offsetting these increases were one-time costs incurred in 2013 associated with the corporate restructuring and listing of our shares of common stock on The NASDAQ Capital Market.

#### Restructuring Costs

We incurred restructuring costs of \$0.3 million and \$1.0 million for the years ended December 31, 2014 and 2013, respectively, related to the closure of our Montreal, Quebec and Princeton, New Jersey facilities. The offices were closed due to the consolidation of our operations to our San Diego facility, with employee separation charges accounting for the majority of the restructuring costs. The restructuring activities associated with the office closures were substantially complete as of March 31, 2014.

#### Change in Fair Value of Warrant Liability

The change in fair value of warrant liability represents expense or income associated with fair value adjustments to the warrant liability recorded during the period. During the year ended December 31, 2014 and 2013, we recorded expense of \$4.5 million and \$19.8 million, respectively, associated with the change in fair value of warrant liability. During the third and fourth quarters of 2014, we amended all of the outstanding warrant agreements to allow for the

warrants to be denominated in U.S. Dollars. As a result of this amendment, the warrants qualify for equity classification and were reclassified into stockholders' equity at their fair value as of the amendment date and revaluations of fair value are no longer required.



## Other Income (Expense), Net

Other income (expense), net consists primarily of interest income and foreign exchange gains and losses. Other income (expense), net for the year ended December 31, 2014 and 2013 was expense of \$0.1 million and \$1.1 million, respectively. The decrease in expense primarily reflects the impact of foreign exchange rates as we operated in U.S. dollars throughout the year ended December 31, 2014.

## Comparison of the Years Ended December 31, 2013 and 2012

The following table summarizes the results of our operations for the years ended December 31, 2013 and 2012 (in thousands):

	Year Ended December 31,		Increase (Decrease)
	2013	2012	
Research and development, net	\$ 19,797	\$ 15,081	\$ 4,716
General and administrative	11,177	5,417	5,760
Restructuring costs	1,025	—	1,025
Other income (expense), net	(1,084)	) 251	(1,335)
Change in fair value of warrant liability	(19,799)	) —	(19,799)

For the year ended December 31, 2013 and 2012, reclassifications were made to certain research and development program expenses line items to conform to the current year presentation. These reclassifications had no impact on total research and development expenses.

## Research and Development Expenses

The following table summarizes our research and development expenses, (in thousands):

	Year Ended December 31,		Increase (Decrease)
	2013	2012	
Third-party development expense:			
MGCD265	\$ 6,588	\$ 7,261	\$ (673)
MGCD516	2,495	—	2,495
mocetinostat	2,580	59	2,521
MGCD290*	1,629	3,477	(1,848)
Total third-party development expense	13,292	10,797	2,495
Internal research and development expense	7,336	5,959	1,377
Research and development expense, gross	20,628	16,756	3,872
Less: Investment tax credits	(831)	) (1,675)	) 844
Research and development expense	\$ 19,797	\$ 15,081	\$ 4,716

\*Development of MGCD290 ceased in early 2013

For the years ended December 31, 2013 and 2012, reclassifications were made to certain research and development program expenses line items to conform prior years' financial information to the current presentation. These reclassifications had no impact on total research and development expenses.

Research and development expenses were \$19.8 million in 2013 compared to \$15.1 million in 2012. The increase of \$4.7 million primarily reflects increased costs for mocetinostat including costs associated with a dose confirmation clinical trial for mocetinostat which commenced in the fourth quarter of 2013, and costs associated with preparation for an Investigational New Drug, or IND, application for MGCD516. During the year ended December 31, 2012 we recorded a favorable adjustment of prior year calculations of ITCs subsequent to the completion of an audit of such ITCs by the provincial tax authority. Partially offsetting these increases were reduced costs for MGCD290 which we are no longer actively pursuing internally.

## General and Administrative Expenses

General and administrative expenses were \$11.2 million in 2013 compared to \$5.4 million in 2012. The increase of \$5.8 million primarily reflects increased costs associated with management changes and one-time costs associated with the listing of our shares of common stock on the NASDAQ Capital Market and the transition to becoming a Delaware corporation.

## Restructuring Costs

In 2013, we incurred restructuring costs of \$1.0 million due to the closing of the Montreal, Quebec and Princeton, New Jersey facilities. Employee separation charges amounted to \$0.9 million while the remainder primarily related to facility charges and asset impairment. Restructuring activities commenced in 2013 and so there is no expense for the year ended December 31, 2012.

## Other Income (Expense), Net

Other income (expense), net was expense of \$1.1 million for the year ended December 31, 2013 compared to income of \$0.3 million for the year ended December 31, 2012. The increase in expense of \$1.4 million primarily reflects the impact of foreign exchange rate changes between the U.S. dollar and Canadian dollar offset by interest income of \$0.2 million.

## Change in Fair Value of Warrant Liability

We reclassified common stock warrants issued in 2011 and 2012 from stockholders' equity to current liability due to a January 1, 2013 change in our functional currency. During the year ended December 31, 2013 we recorded \$19.8 million of expense associated with the change in fair value of warrant liability. Such fair value adjustments were not required during 2012, therefore there is no such expense recorded for the year ended December 31, 2012.

## Liquidity and Capital Resources

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 previous 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, 2014, we had \$29.3 million of cash, cash equivalents and short-term investments compared to \$62.1 million at December 31, 2013. In February 2015, we completed a follow-on offering of common stock for net proceeds of approximately \$48.2 million. We believe that our current cash, cash equivalents and short-term investments together with the net proceeds from the February 2015 common stock offering will fund our currently planned operations through the third quarter of 2016.

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 assure 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,		
	2014	2013	2012
Net cash used in operating activities	(32,748)	) (29,455	) (16,650)
Net cash provided by (used in) investing activities	24,219	(29,470)	) 55
Net cash provided by financing activities	887	54,757	24,813
Increase (decrease) in cash	(7,642)	) (4,168	) 8,218



### Net cash used in operating activities

Net cash used for operating activities was \$32.7 million, \$29.5 million, and \$16.7 million the years ended December 31, 2014, 2013, and 2012, respectively. Cash used in operating activities during 2014 primarily related to our net losses of \$43.7 million, adjusted for non-cash items such as share-based compensation expense of \$7.1 million, the change in fair value of warrant liability of \$4.5 million, amortization of premium on investments of \$0.5 million, and net cash outflows from a change in our operating assets and liabilities of \$1.4 million. Cash used in operating activities during 2013 primarily related to our of net losses of \$52.9 million, adjusted for non-cash items such as the change in fair value of warrant liability of \$19.8 million, change in fair value adjustment of share-based compensation liability of \$1.4 million, shared-based compensation expense of \$1.8 million, and net cash inflows from a change in our operating assets and liabilities of \$0.3 million. Cash used in operating activities during 2012 primarily related to our net losses of \$20.3 million, adjusted for non-cash items such as share-based compensation expense of \$2.0 million, and net cash inflows from a change in our operating assets and liabilities of \$1.4 million.

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

Investing activities consist primarily of purchases, sales and maturities of short-term investments, and to a lesser extent the purchase of property and equipment. Investing activities provided cash of \$24.2 million in 2014, used \$29.5 million in 2013, and provided \$0.1 million in 2012.

### Net cash provided by financing activities

Net cash provided by financing activities was \$0.9 million, \$54.8 million, and \$24.8 million, for the years ended December 31, 2014, 2013, and 2012, respectively. Net cash provided by financing activities for the year ended December 31, 2014 was due to proceeds from exercise of common stock options and warrants of \$0.9 million. Net cash provided by financing activities during 2013 consisted of proceeds from issuance of common stock, net of issuance costs, of \$54.2 million, and proceeds from exercise of common stock options and warrants of \$0.6 million. Net cash provided by financing activities during 2012 consisted primarily of issuance of common stock, net of issuance costs, of \$19.9 million and issuance of warrants, net of issuance costs, of \$4.9 million.

### Contractual Obligations and Commitments

The following table summarizes our contractual obligations and commitments as of December 31, 2014 that will affect our future liquidity (in thousands):

	Year Ended December 31,				
	Total	Less Than 1 year	1 -3 Years	3 -5 Years	More Than 5 Years
Operating lease obligations(1)	\$841	\$223	\$618	\$—	\$—
Total Contractual Obligations	\$841	\$223	\$618	\$—	\$—

(1) In June 2014 we entered into a multi-year non-cancelable building lease for office space in San Diego, California. The lease expires in January 2018.

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, 2014 and 2013, 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 will remain an emerging growth company until the earliest of (1) the end of the fiscal year in which the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the end of the second fiscal quarter, (2) the end of the fiscal year in which we have total annual gross revenue of \$1 billion or more during such fiscal year, (3) the date on which we issue more than \$1 billion in non-convertible debt in a three-year period, or (4) 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 10% change in interest rates were to have occurred on December 31, 2014, 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 concluded that as of March 31, 2013, the Company's disclosure controls and procedures were not effective due to a material weakness in internal control over financial reporting associated with the restatement of the calculation and disclosure of diluted loss per share for the three months ended March 31, 2013. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. Management presented a remediation plan to the Company's Audit Committee and the Audit Committee approved management's remediation plan, which added a new financial reporting process control for the calculation of diluted earnings per share. Management implemented the internal control remediation plan and as of December 31, 2014, management has concluded, through testing, that these controls are operating effectively and the material weakness is considered remediated.

**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, 2014, 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, 2014, 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, 2014 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 2-Recent 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 2015 annual meeting of stockholders. The information required by this item with respect to executive officers appears under Part I of this annual report on Form 10-K under the caption "Business-Executive Officers and Directors."

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 2015 annual meeting of stockholders.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Shareholder 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 2015 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 2015 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 2015 annual meeting of stockholders.

PART IV

Item 15. Exhibits, Financial Statement Schedules

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

	Page(s)
Consolidated Financial Statements	
<u>Report of Independent Registered Public Accounting Firm</u>	<u>54</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.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

Mirati Therapeutics, Inc.

We have audited the accompanying consolidated balance sheet of Mirati Therapeutics, Inc. as of December 31, 2014, and the related consolidated statements of operations and comprehensive loss, changes in stockholders' equity, and cash flows for the year ended December 31, 2014. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, 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. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Mirati Therapeutics, Inc. at December 31, 2014, and the consolidated results of its operations and its cash flows for the year ended December 31, 2014, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

San Diego, CA

March 11, 2015

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of Mirati Therapeutics, Inc.

We have audited the accompanying consolidated balance sheet of Mirati Therapeutics, Inc. as of December 31, 2013, and the related consolidated statements of operations and comprehensive loss, changes in stockholders' equity, and cash flows for each of the two years in the period ended December 31, 2013. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, 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. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Mirati Therapeutics, Inc. at December 31, 2013, and the consolidated results of its operations and its cash flows for each of the two years in the period ended December 31, 2013, in conformity with U.S. generally accepted accounting principles.

Montreal, Canada  
March 17, 2014

/s/Ernst & Young LLP<sup>(1)</sup>

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<sup>(1)</sup>CPA auditor, CA, public accountancy permit no. A120254

## Mirati Therapeutics, Inc.

## CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share data)

	December 31, 2014	2013
<b>ASSETS</b>		
Current assets		
Cash and cash equivalents	\$6,593	\$14,235
Short-term investments	22,710	47,835
Other current assets	3,354	2,145
Total current assets	32,657	64,215
Property and equipment, net	496	322
Other assets	326	—
Total assets	\$33,479	\$64,537
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities		
Accounts payable and accrued liabilities	5,396	5,245
Warrant liability	—	33,407
Total current liabilities	5,396	38,652
Other liability	21	—
Total liabilities	5,417	38,652
Commitments and contingencies		
Stockholders' equity		
Preferred stock, \$0.001 par value, 10,000,000 shares authorized; none issued and outstanding at both December 31, 2014 and December 31, 2013	—	—
Common stock, \$0.001 par value; 100,000,000 authorized; 13,566,726 and 13,446,976 issued and outstanding at December 31, 2014 and December 31, 2013, respectively	14	13
Additional paid-in capital	260,616	214,756
Accumulated other comprehensive income	9,521	9,507
Accumulated deficit	(242,089)	(198,391)
Total stockholders' equity	28,062	25,885
Total liabilities and stockholders' equity	\$33,479	\$64,537

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,		
	2014	2013	2012
Expenses			
Research and development	\$26,071	\$19,797	\$15,081
General and administrative	12,699	11,177	5,417
Restructuring costs	334	1,025	—
Total operating expenses	39,104	31,999	20,498
Loss from operations	(39,104	) (31,999	) (20,498
Other income (expense), net	(77	) (1,084	) 251
Change in fair value of warrant liability	(4,517	) (19,799	) —
Loss before income taxes	(43,698	) (52,882	) (20,247
Income tax benefit (expense)	—	23	(39
Net loss	\$(43,698	) \$(52,859	) \$(20,286
Unrealized gain (loss) on available-for-sale investments	14	(13	) —
Comprehensive loss	\$(43,684	) \$(52,872	) \$(20,286
Basic and diluted net loss per share	\$(3.24	) \$(4.78	) \$(3.00
Weighted average number of shares used in computing net loss per share, basic and diluted	13,483,467	11,057,040	6,762,985

See accompanying notes

Mirati Therapeutics, Inc.

## CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

(in thousands, except share data)

	Common Stock		Common	Additional	Accumulated	Accumulated	Total
	Shares	Amount	Stock Warrants	paid-in capital	other comprehensive income	deficit	stockholders' equity
Balance at January 1, 2012	6,358,253	\$6	\$6,247	\$132,312	\$8,945	\$ (120,205 )	\$ 27,305
Net loss for the year	—	—	—	—	—	(20,286 )	(20,286 )
Share-based compensation expense	—	—	—	2,009	—	—	2,009
Costs of reorganization	—	—	—	(15 )	—	—	(15 )
Issuance of common stock, net of costs	3,593,819	4	—	19,882	—	—	19,886
Issuance of warrants, net of costs	—	—	4,942	—	—	—	4,942
Net exercise of warrants	5,653	—	(36 )	36	—	—	—
Foreign currency translation	—	—	—	—	575	—	575
Balance at December 31, 2012	9,957,725	\$10	\$11,153	\$154,224	\$9,520	\$ (140,491 )	\$ 34,416
Net loss for the year	—	—	—	—	—	(52,859 )	(52,859 )
Share-based compensation expense	—	—	—	1,823	—	—	1,823
Reclassification of warrants	—	—	(11,153 )	—	—	(5,041 )	(16,194 )
Reclassification of stock option liability	—	—	—	1,369	—	—	1,369
Issuance of common stock, net of costs	3,337,500	3	—	54,193	—	—	54,196
Exercise of options for cash	40,534	—	—	540	—	—	540
Exercise of warrants for cash	2,896	—	—	21	—	—	21
Net exercise of warrants	108,321	—	—	2,586	—	—	2,586
Unrealized loss on investments	—	—	—	—	(13 )	—	(13 )
Balance at December 31, 2013	13,446,976	\$13	\$—	\$214,756	\$9,507	\$ (198,391 )	\$ 25,885
Net loss for the year	—	—	—	—	—	(43,698 )	(43,698 )
Reclassification of warrants from liability	—	—	—	36,931	—	—	36,931
Share-based compensation expense	—	—	—	7,050	—	—	7,050
Exercise of options for cash	76,224	1	—	886	—	—	887
Net exercise of warrants	43,526	—	—	993	—	—	993
Unrealized gain on investments	—	—	—	—	14	—	14
Balance at December 31, 2014	13,566,726	\$14	\$—	\$260,616	\$9,521	\$ (242,089 )	\$ 28,062

See accompanying notes





## Mirati Therapeutics, Inc.

## CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Years Ended December 31,		
	2014	2013	2012
Operating activities:			
Net loss	\$(43,698 )	\$(52,859 )	\$(20,286 )
Non-cash adjustments reconciling net loss to operating cash flows			
Depreciation of property and equipment	199	171	123
Amortization of premium on investments	534	—	—
Share-based compensation expense	7,050	1,823	2,009
Loss on disposal of property and equipment	—	40	—
Change in lease incentive liability	—	(70 )	85
Change in fair value of warrant liability	4,517	19,799	—
Change in fair value adjustment of share-based compensation liability	—	1,369	—
Change in restructuring costs	13	—	—
Changes in operating assets and liabilities			
Other current assets	(1,209 )	340	(15 )
Other assets	(326 )	—	—
Accounts payable and accrued liabilities	151	(68 )	1,434
Other liabilities	21	—	—
Cash flows used for operating activities	(32,748 )	(29,455 )	(16,650 )
Investing activities:			
Purchases of short-term investments	(10,468 )	(68,408 )	(29,431 )
Disposal and maturities of short-term investments	35,073	39,138	29,716
Purchases of property and equipment	(386 )	(204 )	(230 )
Proceeds from disposal of property and equipment	—	4	—
Cash flows provided by / (used for) investing activities	24,219	(29,470 )	55
Financing activities:			
Proceeds from issuance of common stock, net of issuance costs	—	54,196	19,886
Proceeds from issuance of warrants, net of issuance costs	—	—	4,927
Proceeds from exercise of common stock options and warrants	887	561	—
Cash flows provided by financing activities	887	54,757	24,813
Increase / (decrease) in cash and cash equivalents	(7,642 )	(4,168 )	8,218
Effect of exchange rate changes on cash and cash equivalents	—	—	303
Cash and cash equivalents, beginning of year	14,235	18,403	9,882
Cash and cash equivalents, end of year	\$6,593	\$14,235	\$18,403
Supplemental disclosures of non-cash investing and financing activities:			
Income taxes paid	\$—	\$35	\$34
Net exercise of warrants	\$993	\$2,586	\$—

See accompanying notes

Mirati Therapeutics, Inc.  
Notes to Consolidated Financial Statements  
December 31, 2014

1. Description of Business

Mirati Therapeutics, Inc. ("Mirati" or the "Company") is a clinical-stage biopharmaceutical company focused on developing a pipeline of targeted oncology products. The Company focuses its development programs on drugs intended to treat specific genetically defined and selected subsets of cancer patients with unmet needs. 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"). MethylGene's common stock was listed on the Toronto Stock Exchange from June 29, 2004 until July 26, 2013 under the ticker symbol "MYG". The Company also has an indirect, wholly-owned subsidiary, MethylGene US Inc., which was incorporated in Princeton, New Jersey on December 20, 2011 and started business activity in 2012. MethylGene US Inc. ceased operations effective January 1, 2014. During the first half of 2013, the Company conducted the majority of its operations through MethylGene and MethylGene US Inc. As a result of the arrangement agreement discussed in Note 2 under the heading "Basis of Presentation," Mirati became the parent company in June 2013 and primary operating company during the last half of 2013. Refer to Note 2 for further discussion of the Company's corporate structure.

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 ("GAAP"). These consolidated financial statements include the accounts of the Company, MethylGene and MethylGene US Inc. 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. The Company was created to enter into an arrangement agreement described below.

On May 8, 2013, the Company's Board of Directors approved and the Company entered into an arrangement agreement with MethylGene. Subject to the terms and conditions of the arrangement agreement, which was consummated on June 28, 2013, the shareholders of MethylGene received one share of the Company's common stock in exchange for every 50 common shares of MethylGene, which had the effect of a 50 for 1 reverse split of the common shares pursuant to a court-approved plan of arrangement under Section 192 of the Canada Business Corporations Act. Such transaction is referred to herein as the Arrangement. In addition, all outstanding options and warrants to purchase common shares of MethylGene became exercisable on a 50-for-1 basis for shares of our common stock, and a proportionate adjustment was made to the exercise price or conversion price, as applicable. The accompanying financial statements and notes to the financial statements give retroactive effect to the reverse split of our common stock for all periods presented. Upon completion of the Arrangement, MethylGene became the Company's wholly-owned subsidiary. The shares of the Company's common stock issued at the closing of the Arrangement were issued in reliance upon the exemption from registration under Section 3(A)(10) of the Securities Act of 1933, as amended.

These consolidated financial statements are presented in U.S. dollars, which effective January 1, 2013, is also the functional currency of the Company.

Use of Estimates

The preparation of the Company's audited consolidated financial statements 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 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.

#### Foreign Currency Transactions

Foreign currency transactions are initially recorded by the Company using the exchange rates prevailing at the date of the transaction. At the balance sheet date, monetary assets and liabilities denominated in foreign currencies are translated at the period-end rates of exchange. Non-monetary assets and liabilities are translated at the historical exchange rates. Exchange gains and losses arising from the translation of foreign currency items are included in other income (expense) in the consolidated statements of operations and comprehensive loss. The Company recognized net foreign exchange losses of \$0.2 million, \$1.3 million and an immaterial amount in other income (expense) in the consolidated statement of operations and comprehensive loss for the years ended December 31, 2014, 2013 and 2012, respectively.

#### Property and Equipment

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

On 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 immaterial impairment charges related to property and equipment for the years ended December 31, 2014 and 2013, and no impairment charges in 2012.



#### Reclassification of Warrants

In 2011 and 2012, MethylGene issued common stock warrants in connection with the issuance of common stock through private placements (referred to as the 2011 Warrants and the 2012 Warrants). The exercise prices of the 2011 and 2012 Warrants were denominated in Canadian dollars. Upon the issuance of the 2011 and 2012 Warrants, the net proceeds were allocated to common stock and warrants based on their relative fair values, and the fair value of the issued common stock warrants was calculated utilizing the Black-Scholes option-pricing model. The allocated fair value was then recorded as warrants within stockholders' equity on the consolidated balance sheet.

Effective January 1, 2013, the Company changed its functional currency which changed how the 2011 and 2012 warrants are accounted for as they continued to have exercise prices denominated in Canadian dollars. Upon the change in functional currency, the warrants were classified as a current liability and a warrant liability of \$16.2 million which represented the fair market value of the warrants at that date in accordance with accounting standards. The initial fair value recorded as warrants within stockholders' equity of \$11.2 million was reversed. The change in fair value related to periods prior to January 1, 2013 of \$5.0 million was recorded as an adjustment to accumulated deficit. At each reporting period subsequent to January 1, 2013, the fair value of the warrant liability was recalculated and any corresponding increase or decrease to the warrant liability was recorded as change in fair value of warrant liability on the consolidated statement of operations and comprehensive loss. The estimated fair value was determined using the Black-Scholes option-pricing model based on the estimated value of the underlying common stock at the valuation measurement date, the remaining contractual term of the warrants, risk-free interest rates, expected dividends and expected volatility of the price of the underlying common stock.

During the second half of 2014, the Company amended all of its outstanding warrant agreements to allow for the warrants to be denominated in U.S. Dollars. As a result of this amendment, the amended warrants qualified for equity classification and were reclassified into stockholders' equity at their fair value as of the amendment date and revaluations of fair value are no longer required.

Prior to the amendments, for all of the warrants classified as liabilities during the relevant periods, the Company recorded warrant valuation expense of \$4.5 million and \$19.8 million for the years ended December 31, 2014 and 2013, respectively. No such expense was recorded for the year ended December 31, 2012 as the functional currency was the Canadian dollar.

#### Reclassification of Share-Based Compensation Liability

The Company granted stock options denominated in Canadian dollars under its 1997 Equity Plan to Canadian and United States, or US, based employees and directors until July 26, 2013. Following the delisting of the Company's shares from the Toronto Stock Exchange, the options denominated in Canadian dollars that were granted to US-based employees and US-based directors were subject to liability accounting ("liability options") with fair value calculated using the Black-Scholes option-pricing model. The Company revalued the liability options as of July 26, 2013 and recorded a share-based compensation liability of \$1.1 million with a corresponding reduction of additional paid-in capital.

At each reporting period subsequent to July 26, 2013, the Company adjusted the fair value of the liability options and any corresponding increase or decrease to the liability was recorded as either a reduction of additional paid in capital or as stock compensation expense on the consolidated statement of operations and comprehensive loss, as appropriate. During the year ended December 31, 2013 these fair value adjustments resulted in an increase to additional paid in capital of \$0.3 million and total stock compensation expense of \$1.4 million. Effective November 30, 2013 the Company amended the agreements underlying the liability options such that the exercise price was converted from Canadian dollars to the equivalent US dollar exercise price by applying the exchange rate for the conversion of

Canadian dollars into U.S. dollars based on the Bank of Canada's noon buying rate for one U.S. dollar on the date the option was granted. The fair value of the liability options as of November 30, 2013 was \$2.2 million and was reclassified from share-based compensation liability to additional paid-in capital.

#### Share-Based Compensation

The Company has a stock option compensation plan in which the fair value of stock options granted is determined at the date of the grant using the Black-Scholes option-pricing model and is expensed over the vesting period of the options. Share-based compensation is recognized using the graded accelerated vesting method. In determining the expense, the Company deducts the

number of options that are expected to be forfeited at the time of a grant and revises this estimate, if necessary, in subsequent years if actual forfeitures differ from those estimated. The stock-based compensation expense attributable to awards under the Company's 2013 Employee Stock Purchase Plan ("ESPP") was also determined using the Black-Scholes option pricing model.

The determination of the fair value of share-based compensation awards utilizing the Black-Scholes model is affected by the Company's stock price and a number of assumptions, including but not limited to expected stock price volatility over the term of the awards and the expected term of stock options. Changes in the assumptions can materially affect the fair value estimates.

#### 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, said ITCs are applied as a reduction of tax expense. As operations in Canada ceased in early 2014, there were no new investment tax credits earned for the year ended December 31, 2014.

#### 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: salaries and benefits, share-based compensation expense, allocated overhead and occupancy costs, clinical trial and related clinical manufacturing costs, contract services, and other outside costs.

#### 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 income in the period that includes the enactment date. A valuation allowance against deferred tax assets is recorded if, based upon the weight of all available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. For uncertain tax positions that meet "a more likely than not" threshold, the Company recognizes the benefit of uncertain tax positions in the consolidated financial statements.

#### Segment Reporting

Operating segments are components of an enterprise about which 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 has viewed its operations and managed its business as one segment operating primarily in the United States.

#### 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;

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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, 2014	Level 1	Level 2	Level 3
Assets				
Cash and cash equivalents	\$6,593	\$4,590	\$2,003	\$—
Short-term investments	22,710	—	22,710	—
	\$29,303	\$4,590	\$24,713	\$—
	December 31, 2013	Level 1	Level 2	Level 3
Assets				
Cash and cash equivalents	\$14,235	\$12,431	\$1,804	\$—
Short-term investments	47,835	—	47,835	—
	\$62,070	\$12,431	\$49,639	\$—
Liabilities				
Warrant liability	\$33,407	\$—	\$—	\$33,407
	\$33,407	\$—	\$—	\$33,407

The Company's investments in Level 1 assets are valued based on publicly available quoted market prices for identical securities as of December 31, 2014 and December 31, 2013. 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, 2014 and 2013.

The following table presents a rollforward of the fair value of the warrant liability, which included Level 3 measurements (in thousands):

	Fair Value Measurements at Reporting Date Using Significant Unobservable Inputs (Level 3)
Warrant liability:	
Balance at January 1, 2013	\$—
Fair value upon reclassification of balance as of January 1, 2013	16,194
Change in fair value of warrant liability included in net loss	19,799
Fair value of warrants exercised	(2,586 )
Balance at December 31, 2013	33,407
Change in fair value of warrant liability included in net loss	4,517
Fair value of warrants exercised	(993 )
Reclassification of warrants to stockholders' equity	(36,931 )
Balance at December 31, 2014	\$—

The Company estimated the fair value of warrants at the time of issuance and subsequent remeasurement through the date of reclassification into equity using the Black-Scholes option-pricing model at each reporting date, using the following inputs: the risk-free interest rates; the expected dividend rates; the remaining expected life of the warrants; and the expected volatility of the price of the underlying common stock. The estimates are based, in part, on subjective assumptions and changes to these assumptions could have a significant impact on the fair value of the warrants.

The following assumptions were used in the Black-Scholes option-pricing model as of the warrant amendments dates to determine the fair value of the warrants to reclassify into equity:

	September 2014				December 2014	
	2011 Warrants		2012 Warrants		2011 Warrants	
Risk-free interest rate	1.2	%	1.2	%	1.0	%
Volatility	108.6	%	100.8	%	62.8% - 63.9%	
Dividend yield	—		—		—	
Expected life in years	1.6		3.2		1.3	

The following assumptions were used in the Black-Scholes option-pricing model to determine the fair value of the warrant liability as of December 31, 2013. All 2011 and 2012 warrants were amended and did not require a valuation as of December 31, 2014.

	December 31, 2013			
	2011 Warrants		2012 Warrants	
Risk-free interest rate	1.2	%	1.6	%
Volatility	112.0	%	115.9	%
Dividend yield	—		—	
Expected life in years	2.3		3.9	

### 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 December 31,		
	2014	2013	2012
Common stock options	253,595	495	15,663
Common stock warrants	1,515,445	644,426	690,046
Total	1,769,040	644,921	705,709

### 3. Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB") or other standard setting bodies that are adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on our consolidated financial position or results of operations upon adoption.

In May 2014, the FASB issued Accounting Standard Update ("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. The new standard will be effective for annual reporting periods beginning after December 15, 2016, including interim periods within that reporting period. The Company is currently evaluating the impact that this standard will have on its consolidated financial statements.

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 will be 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 beginning after December 15, 2016, and for annual and interim periods thereafter, early adoption is permitted. The Company has not elected to early adopt and is currently evaluating the potential changes from this ASU to its future financial reporting and disclosures.

### 4. Investments

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

		As of December 31, 2014			
	Maturity (in years)	Amortized cost	Gross unrealized gains	Gross unrealized losses	Estimated fair value
Corporate debt securities (1)	1 year or less	21,208	3	(1	) 21,210
Commercial paper (1)	1 year or less	1,500	—	—	1,500
		22,708	3	(1	) 22,710
		As of December 31, 2013			
	Maturity (in years)	Amortized cost	Gross unrealized gains	Gross unrealized losses	Estimated fair value
Government sponsored enterprise (1)	2 years or less	3,001	—	(3	) 2,998
Corporate debt securities (1)	2 years or less	31,319	—	(22	) 31,297
Commercial paper (1)	1 year or less	8,485	12	—	8,497
Guaranteed investment certificates (2)	1 year or less	5,046	—	(3	) 5,043
		47,851	12	(28	) 47,835

(1) Investments are designated as available-for-sale investments.

(2) Investments are designated as trading investments.

Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At December 31, 2014, 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. Other Current Assets

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

	December 31,	
	2014	2013
	(in thousands)	
Prepaid expenses	1,827	667
Refundable research and development tax credits	809	809
Security deposits and other receivables	622	506
Interest receivables	96	163
	\$3,354	\$2,145

## 6. Property and Equipment, Net

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

	December 31, 2014	2013
	(in thousands)	
Computer equipment	\$329	\$1,534
Office and other equipment	56	122
Laboratory equipment	346	1,794
Leasehold improvements	—	53
	\$731	\$3,503
Less: Accumulated depreciation	(235)	(3,181)
	\$496	\$322

The Company incurred depreciation expense of \$0.2 million, \$0.2 million and \$0.1 million for the years ended December 31, 2014, 2013 and 2012, respectively. During the year ended December 31, 2014, in connection with the Company's closure of its Canadian office as described further in Note 8, the Company disposed of several fully depreciated assets with a gross book value of \$3.2 million that were no longer in use.

## 7. Accounts Payable and Accrued Liabilities

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

	December 31, 2014	2013
	(in thousands)	
Accounts payable	\$1,655	\$1,302
Accrued expenses	2,327	2,335
Accrued compensation and benefits	1,414	1,608
	\$5,396	\$5,245

## 8. Restructuring

On October 1, 2013, the Company announced a plan to terminate approximately 75% of its total workforce in connection with the closing of its Montreal, Quebec and Princeton, New Jersey offices (the "Restructuring") due to the consolidation of Company operations to the San Diego facility. Restructuring costs were comprised of employee separation and facilities closure related costs and were reported as a separate line item in the accompanying consolidated statement of operations and comprehensive loss. A rollforward of the accrued restructuring liability is presented below (in thousands):

Balance as of October 1, 2013	\$—	
Accrued restructuring charges	1,025	
Payments	(729)	)
Balance as of December 31, 2013	296	
Accrued restructuring charges	334	
Payments	(630)	)
Balance as of December 31, 2014	\$—	

The restructuring activities associated with the office closures were substantially complete as of March 31, 2014, and all restructuring costs incurred have been paid to date.

## 9. Stockholders' Equity

### Common Stock

As of December 31, 2014, the following shares were reserved for future issuance:

	December 31, 2014
Common stock options outstanding and available for future grant	1,891,107
Warrants to purchase common stock	2,465,713
Employee Stock Purchase Plan	300,000
	4,656,820

### Warrants

As of December 31, 2014 the following warrants for common stock were issued and outstanding:

Issue date	Expiration date	Exercise price (Denominated in US Dollars)	Number of warrants outstanding
April 4, 2011	April 4, 2016	\$6.74	1,397,921
November 21, 2012	November 21, 2017	\$7.86	1,067,792
			2,465,713

On April 4, 2011 and November 21, 2012 the Company's wholly owned subsidiary, MethylGene, completed private placement financing transactions which resulted in the issuance of common stock warrants. The April 4, 2011 common stock warrants were issued with an exercise price of CND\$7.46 and the November 21, 2012 common stock warrants were issued with an exercise price of CND\$8.70. During the last half of 2014, the Company amended all of these outstanding warrant agreements to allow for the warrants to be denominated in U.S. Dollars.

During the year ended December 31, 2014, warrants for 73,964 shares of the Company's common stock were exercised via cashless exercises and the Company issued a total of 43,526 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. 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, 2014, there were 0.4 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, 2014:

	Number of options	Weighted average exercise price	Weighted-Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (millions)
Balance, December 31, 2013	968,923	\$11.13		
Granted	599,410	\$18.64		
Exercised	(76,224 )	\$11.20		
Canceled/forfeited	(34,640 )	\$15.44		
Expired	(2,609 )	\$55.80		
Balance, December 31, 2014	1,454,860	\$14.00		
Options exercisable at December 31, 2014	431,088	\$12.25	7.0	\$3.21
Options vested and expected to vest at December 31, 2014	1,383,082	\$14.89	8.1	\$6.83

The total intrinsic value of stock options exercised was \$0.6 million and \$0.2 million for the years ended December 31, 2014 and 2013, respectively. The Company received total cash of \$0.9 million and \$0.5 million for the exercise of options for the years ended December 31, 2014 and 2013. There were no options exercised during the year ended December 31, 2012. The total fair value of options vested during the years ended December 31, 2014, 2013, and 2012 was \$3.0 million, \$2.0 million, and \$2.4 million respectively. Upon option exercise, the Company issues new shares of our common stock.

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

	Year ended December 31,		
	2014	2013	2012
Research and development expense	\$2,565	\$245	\$817
General and administrative expense	4,485	2,947	1,192
	\$7,050	\$3,192	\$2,009

In the years ended December 31, 2014, 2013 and 2012, 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,		
	2014	2013	2012
Risk-free interest rate	2.1%	2.0%	1.2%
Dividend yield	—%	—%	—%
Volatility factor	113.0%	112.9%	116.4%
Expected term (in years)	6.7	7.0	4.4
Weighted average estimated fair value per share	\$16.09	\$9.75	\$9.00

**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 either the Canadian Treasury yield (for grants prior to July 16, 2013), or U.S. Treasury zero-coupon bonds (for grants after July 16, 2013).

**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. Prior to the fourth quarter of 2013, the Company estimated the life of the options using historical data as well as various assumptions





regarding the Company's expected progress on its development programs. As a result of the Arrangement, beginning in the fourth quarter of 2013, the Company began to use 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 more appropriate as the Company's historical stock option activity is no longer predictive of future activity due to the Arrangement, the Company's listing on NASDAQ and subsequent delisting from the TSX and the organizational changes announced on October 1, 2013.

The total compensation cost not yet recognized as of December 31, 2014 related to non-vested option awards was \$7.1 million which will be recognized over a weighted-average period of 1.5 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, 2014, no shares have yet 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. During the years ended December 31, 2014, expense associated with the Company's matching contribution totaled \$0.1 million and an immaterial amount for the years ended December 31, 2013 and 2012, respectively.

### 12. Income Taxes

The Company's provisions for income tax benefit (expense) are as follows (in thousands):

	Year ended December 31,		
Current:	2014	2013	2012
Federal	\$—	\$23	\$(30)
State	—	—	(9)
Canada	—	—	—
Total current tax expense (benefit)	\$—	\$23	\$(39)

## Tax Expense or Benefit

The differences between the effective income tax rate and the statutory tax rates during the years ended 2014, 2013 and 2012 are as follows (in thousands):

	Year Ended December 31,		
	2014	2013	2012
Net loss before tax	\$(43,698 )	\$(52,882 )	\$(20,247 )
Statutory combined US federal and state tax rate (2012 - statutory combined Canadian federal and provincial tax rate)	39.83 %	39.83 %	26.90 %
Statutory federal and provincial taxes	\$(17,405 )	\$(21,063 )	\$(5,446 )
Increase (decrease) in taxes recoverable resulting from:			
Effect of change in valuation allowance	12,273	8,537	5,145
Non-deductible share-based compensation	930	1,085	539
Non-deductible warrant expenses for tax purposes	1,799	8,403	—
Tax credits	(180 )	(96 )	(70 )
Share issue costs - temporary difference	(184 )	(184 )	(183 )
Share issue costs - permanent difference	—	206	—
Effect of foreign jurisdiction tax expense	—	—	39
Differential in income tax rates of foreign subsidiary	3,047	3,059	—
Other differences	(280 )	30	15
Income tax expense (benefit)	\$—	\$(23 )	\$39

The combined statutory tax rate used for fiscal 2014 and 2013 differs from the previous year due to the change in home jurisdiction under the consolidation process pursuant to the plan of arrangement agreement, which was consummated on June 28, 2013.

## Deferred Tax

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

	December 31,	
	2014	2013
Deferred tax assets:		
Tangible and intangible depreciable assets	\$185	\$874
Stock compensation	2,360	—
Manufactured drug product inventory to be used in research	1,425	1,459
Provisions	554	93
Financing fees	261	445
Net operating loss carry forwards	23,243	13,099
Scientific research and experimental development expenditures	5,715	5,766
Research and development tax credits	266	—
Total gross deferred tax assets	34,009	21,736
Less valuation allowance	(34,009 )	(21,736 )
Net deferred tax assets	\$—	\$—

Total valuation allowance increased by \$12.3 million for the year ended December 31, 2014. The Company has determined that it is more likely than not that it will not recognize the benefits of its US federal and state deferred tax assets and its Canadian

federal and provincial deferred tax assets and, as a result, has established a full valuation allowance against its deferred tax assets as of December 31, 2014.

For Canadian federal income tax purposes, the Company's Canadian federal scientific research and experimental development expenditures amounted to \$20.1 million, \$20.7 million and \$15.2 million for the years ended December 31, 2014, 2013 and 2012, respectively and for provincial income tax purposes amounted to \$22.7 million, \$22.4 million and \$16.8 million for the years ended December 31, 2014, 2013 and 2012, respectively. As operations in Canada ceased in during 2014, the expenditures incurred for the year ended December 31, 2014 were much lower than previous years. 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.

The Company also has accumulated share issue expenses that have not been deducted for income tax purposes amounting to approximately \$1.0 million, \$1.7 million and \$2.4 million for the years ended December 31, 2014, 2013 and 2012, respectively. The benefits of these expenses have not been recognized in the financial statements.

The Company's net operating loss carry forwards, or NOLs, for US federal and state income taxes were \$10.3 million and \$9.5 million, respectively, for the year ended December 31, 2014. In addition, the Company has research and development tax credit carryforwards for federal and state income tax purposes as of December 31, 2014 of \$0.2 million and \$0.1 million, respectively. The Company's NOLs for Canadian federal and provincial income tax purposes, were \$71.6 million and \$71.0 million, respectively, for the year ended December 31, 2014.

The NOLs are available to offset future taxable income from US federal and state tax sources and 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 Federal	State	Canada Federal	Provincial
Expires in:				
2030	\$—	\$—	\$5,907	\$5,985
2031	—	—	7,059	7,066
2032	—	—	13,312	12,433
2033	3,261	2,286	18,623	19,385
2034	7,012	7,185	26,741	26,149
	\$10,273	\$9,471	\$71,642	\$71,018

The future utilization of the US federal and state NOLs 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, or the 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 ownerships in excess of 50% as defined in the Act. The Canadian Federal and Provincial Tax Acts maintain similar rules in the case of acquisition of control. The Company files income tax returns in the US (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.

The Company's Canadian operations have been audited for provincial tax purposes up to and including December 31, 2009. For Canadian federal tax purposes, the Company remains subject to audit for the December 31, 2010 and subsequent taxation 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 December 31,			Provincial/State December 31,		
	2014	2013	2012	2014	2013	2012
Unrecognized tax positions, beginning of year	\$35	\$43	\$42	\$6	\$2	\$1
Gross decrease — current period tax positions	—	—	—	—	—	—
Gross increase — current period tax positions	35	—	1	12	—	1
Gross decrease — prior period tax positions	(28	) (13	) —	—	—	—
Gross increase — prior period tax positions	—	5	—	—	4	—
Unrecognized tax positions, end of year	\$42	\$35	\$43	\$18	\$6	\$2

Included in the balance of unrecognized tax positions at December 31, 2014 is \$0.1 million and an immaterial amount for 2013 and 2012, that if recognized, would not 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 at December 31, 2014 and 2013 and the Company had no ongoing tax audits as of December 31, 2014.

### 13. Investment Tax Credits

The Company is eligible to claim Canadian federal and provincial ITCs for eligible scientific research and development expenditures. The Company records 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 recorded provincial refundable ITCs as a reduction of research and development expenditures of \$0.9 million and \$1.7 million (including a \$1.1 million favorable adjustment resulting from a statutory audit), for the years ended December 31, 2013 and 2012, respectively. The Company has an ongoing audit related to the provincial refundable ITCs for the year ended December 31, 2013. The Company did not record provincial refundable ITCs as a reduction of research and development expenditures for the year ended December 31, 2014 because 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, 2014 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 reduction of tax expense when realized.

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

	FEDERAL 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 will serve as the Company's new corporate headquarters, replacing the current facilities. The lease will commence in three phases, with 2,300 square feet of space which commenced on July 1, 2014 at an initial monthly rent of approximately \$5,900 per month, 14,000 square feet of space becoming available in the first quarter of 2015 at an initial monthly rent of \$14,000 per month, and the final 1,600 square feet of space becoming available in the first quarter of 2016 at an initial monthly rent of approximately \$4,200 per month. Each portion of the leased property will be subject to a 3% annual rent increase following availability. In addition to such base monthly rent, the Company will be obligated to pay triple net lease charges for operating expenses, taxes, insurance and utilities applicable to the leased property. The lease will expire on January 31, 2018 with respect to the entire premises. Future minimum payments required under the lease are summarized as follows (in thousands):

##### Year Ending December 31:

2015	\$223
2016	290
2017	303
2018	25
Total minimum lease payments	\$841

Total lease expense for the years ended December 31, 2014, 2013 and 2012 was \$0.4 million, \$0.4 million, and \$0.3 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, 2014 and 2013 (unaudited, in thousands, except for per share data):

	Quarted Ended				Year Ended
2014:	First	Second	Third	Fourth	December 31,
Operating Loss	\$(7,979 )	\$(10,134 )	\$(10,548 )	\$(10,443 )	\$(39,104 )
Net loss	\$(13,656 )	\$(11,038 )	\$(8,617 )	\$(10,387 )	\$(43,698 )
Per common share:					
Loss per share, basic	\$(1.01 )	\$(0.82 )	\$(0.64 )	\$(0.77 )	\$(3.24 )
Loss per share, diluted	\$(1.01 )	\$(0.82 )	\$(0.72 )	\$(0.77 )	\$(3.24 )

	Three Months Ended				Year Ended
2013:	First	Second	Third	Fourth	December 31,
Operating Loss	\$(8,006 )	\$(6,898 )	\$(9,209 )	\$(7,886 )	\$(31,999 )
Net loss	\$(4,217 )	\$(8,012 )	\$(29,398 )	\$(11,232 )	\$(52,859 )
Per common share:					
Loss per share, basic	\$(0.42 )	\$(0.80 )	\$(2.95 )	\$(0.96 )	\$(4.78 )
Loss per share, diluted	\$(0.68 )	\$(0.80 )	\$(2.95 )	\$(0.96 )	\$(4.78 )

#### 16. Subsequent Events

##### Sale of Common Stock

In February 2015, the Company completed a follow-on offering whereby it issued an aggregate 2,587,500 shares of common stock at \$20.00 per share. Proceeds from the follow-on offering, net of underwriting discounts, commissions and offering expenses, were approximately \$48.2 million.



## 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 11, 2015

by: /s/ Charles M. Baum, M.D., Ph.D.  
Chief Executive Officer

Date: March 11, 2015

by: /s/ Mark J. Gergen  
Executive Vice President and  
Chief Operations 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 Mark J. Gergen 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 Charles M. Baum, M.D., Ph.D.	Chief Executive Officer and Director (Principal Executive Officer)	March 11, 2015
/S/ MARK J. GERGEN Mark J. Gergen	Executive Vice President, Chief Operations Officer (Principal Financial Officer)	March 11, 2015
/S/ JAMIE A. DONADIO Jamie A. Donadio	Vice President, Finance (Principal Accounting Officer)	March 11, 2015
/S/ RODNEY LAPPE Rodney Lappe, Ph.D.	Chairman of the Board	March 11, 2015
/S/ MICHAEL GREY Michael Grey	Director	March 11, 2015
/S/ HENRY J. FUCHS Henry J. Fuchs, M.D.	Director	March 11, 2015
/S/ CRAIG JOHNSON Craig Johnson	Director	March 11, 2015
/S/ WILLIAM R. RINGO William R. Ringo	Director	March 11, 2015

## INDEX TO EXHIBITS

Exhibit number	Description of document
2.1	Arrangement Agreement, dated May 8, 2013, by and between MethylGene Inc. and the Registrant. <sup>(2)</sup>
3.1	Amended and Restated Certificate of Incorporation. <sup>(1)</sup>
3.2	Bylaws. <sup>(1)</sup>
4.1	Form of Common Stock Certificate. <sup>(2)</sup>
10.1	Form of Securities Purchase Agreement relating to the 2011 private placement. <sup>(1)</sup>
10.2	Form of Securities Purchase Agreement relating to the 2012 private placement. <sup>(1)</sup>
10.3	Form of Warrant Certificate issued in connection with the 2011 private placement. <sup>(1)</sup>
10.4	Form of Warrant Certificate issued in connection with the 2012 private placement. <sup>(1)</sup>
10.5+	Amended and Restated Incentive Stock Option Plan. <sup>(1)</sup>
10.6+	Form of 2013 Equity Incentive Plan and Form of Stock Option Grant Notice and Form of Stock Option Agreement thereunder. <sup>(1)</sup>
10.7+	Form of 2013 Employee Stock Purchase Plan. <sup>(1)</sup>
10.12	Collaboration and License Agreement, dated October 16, 2003, by and between MethylGene Inc. and Taiho Pharmaceutical Co. Ltd.
10.13	Amendment Number One to Collaboration and License Agreement, dated January 25, 2005, by and between MethylGene Inc. and Taiho Pharmaceutical Co., Ltd.
10.14	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.
10.15+	Senior Executive Employment Agreement, dated September 24, 2012, by and among MethylGene Inc. and Dr. Charles M. Baum. <sup>(1)</sup>
10.16+	Employment Agreement, dated February 15, 2013, by and between MethylGene Inc. and Mark J. Gergen. <sup>(1)</sup>
10.17+	Amended and Restated Employment Agreement, dated July 2, 2013, by and between the Registrant and Dr. Charles M. Baum. <sup>(3)</sup>
10.18+	Amended and Restated Employment Agreement, dated July 2, 2013, by and between the Registrant and Mark J. Gergen. <sup>(3)</sup>
10.19	Sublease Agreement, dated May 28, 2013, by and between Amylin Pharmaceuticals, LLC and MethylGene US, Inc. <sup>(4)</sup>
10.2	Lease Agreement, dated June 24, 2014, by and between the Company and ARE-SD Region No. 20, LLC. <sup>(6)</sup>
10.21+	Letter Agreement, dated August 30, 2013, by and between the Registrant and Dr. Isan Chen. <sup>(5)</sup>
10.22+	Letter Agreement, Dated May 20, 2013, by and between Methylgene Inc. and James Christensen
10.23+	Form of Indemnity Agreement. <sup>(5)</sup>
21.1	Subsidiaries of the Registrant. <sup>(1)</sup>
23.1	Consent of Independent Registered Public Accounting Firm- US.
23.2	Consent of Independent Registered Public Accounting Firm- Canada.
31.1	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.
101.CAL	XBRL 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.

<sup>+</sup> Indicates management contract or compensatory plan.

<sup>\*</sup> 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2013, filed with the Securities and Exchange Commission on August 13, 2013.
- (5) 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.
- (6) 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.