

Mirati Therapeutics, Inc.
Form 10-K
March 11, 2015
UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

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

MIRATI THERAPEUTICS, INC.
(Exact Name of Registrant as Specified in Its Charter)

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

9363 Towne Centre Drive Suite 200, San Diego, 92121
California (Zip Code)
(Address of principal executive offices)

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)

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 Accelerated filer
Non-accelerated filer (Do not check if a smaller reporting company) 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.

Table of Contents

	Page
PART I	
Item 1. Business	3
Item 1A. Risk Factors	<u>18</u>
Item 1B. Unresolved Staff Comments	<u>40</u>
Item 2. Properties	<u>40</u>
Item 3. Legal Proceedings	<u>40</u>
Item 4. Mine Safety Disclosures	<u>40</u>
PART II	
Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	<u>40</u>
Item 6. Selected Consolidated Financial Data	<u>42</u>
Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations	<u>43</u>
Item 7A. Quantitative and Qualitative Disclosures About Market Risk	<u>51</u>
Item 8. Financial Statements and Supplementary Data	<u>51</u>
Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure	<u>51</u>
Item 9A. Controls and Procedures	<u>51</u>
Item 9B. Other Information	52
PART III	
Item 10. Directors, Executive Officers and Corporate Governance	<u>52</u>
Item 11. Executive Compensation	<u>52</u>
Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	<u>52</u>
Item 13. Certain Relationships and Related Transactions, and Director Independence	<u>52</u>
Item 14. Principal Accountant Fees and Services	<u>52</u>
PART IV	
Item 15. Exhibits and Financial Statement Schedules	<u>53</u>
SIGNATURES	<u>77</u>

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 potently 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. 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 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.

6

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.

7

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.

8

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 Zolinza 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

10

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

11

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. ⁽³⁾	60	Chairman of the Board
Michael Grey ⁽¹⁾⁽³⁾	62	Director
Henry J. Fuchs, M.D. ⁽²⁾⁽³⁾	55	Director
Craig Johnson ⁽¹⁾⁽²⁾	53	Director
William R. Ringo ⁽¹⁾⁽²⁾	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,

29

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;

33

- 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

40

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.